UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K
CURRENT REPORT
Pursuant to Section 13 or 15(d) of
The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): June 4, 2025

Biohaven Ltd.
(Exact name of registrant as specified in its charter)

British Virgin Islands	**001-41477**	**Not applicable**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

c/o Biohaven Pharmaceuticals, Inc.
215 Church Street
New Haven, Connecticut 06510
(Address of principal executive offices, including zip code)
(203) 404-0410
(Registrant's telephone number, including area code)
Not applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol	Name of each exchange on which registered
Common Shares, no par value	BHVN	New York Stock Exchange

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure

On June 4, 2025, Biohaven Ltd. will be making an investor presentation (the "Presentation"). A copy of the Presentation is attached as Exhibit 99.1 to this Current Report on Form 8-K, and is incorporated herein by reference.

The information in this Current Report on Form 8-K, including the information set forth in Exhibit 99.1, is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Exhibit Description
99.1	Investor Presentation, dated June 2025.
104	The cover page of this Current Report on Form 8-K formatted as Inline XBRL.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: June 4, 2025

Biohaven Ltd.

By: /s/ Matthew Buten
Matthew Buten
Chief Financial Officer

biohaven®

DAYS MATTER™

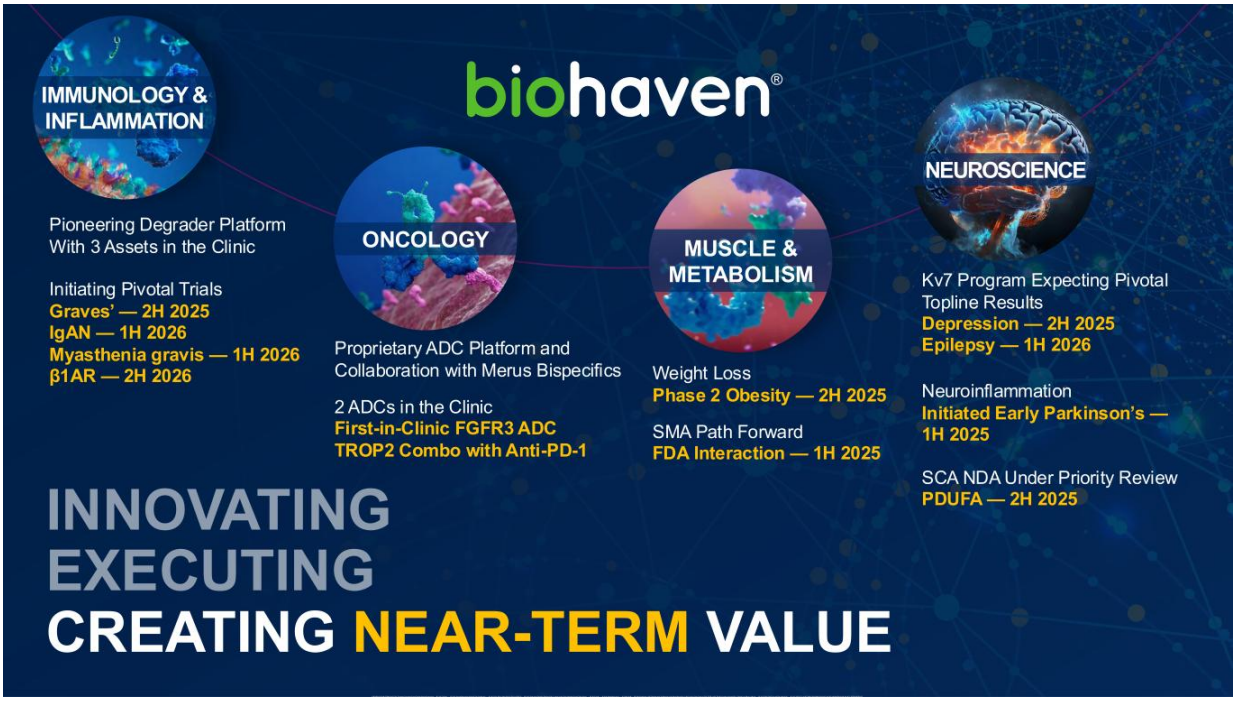
Corporate Presentation

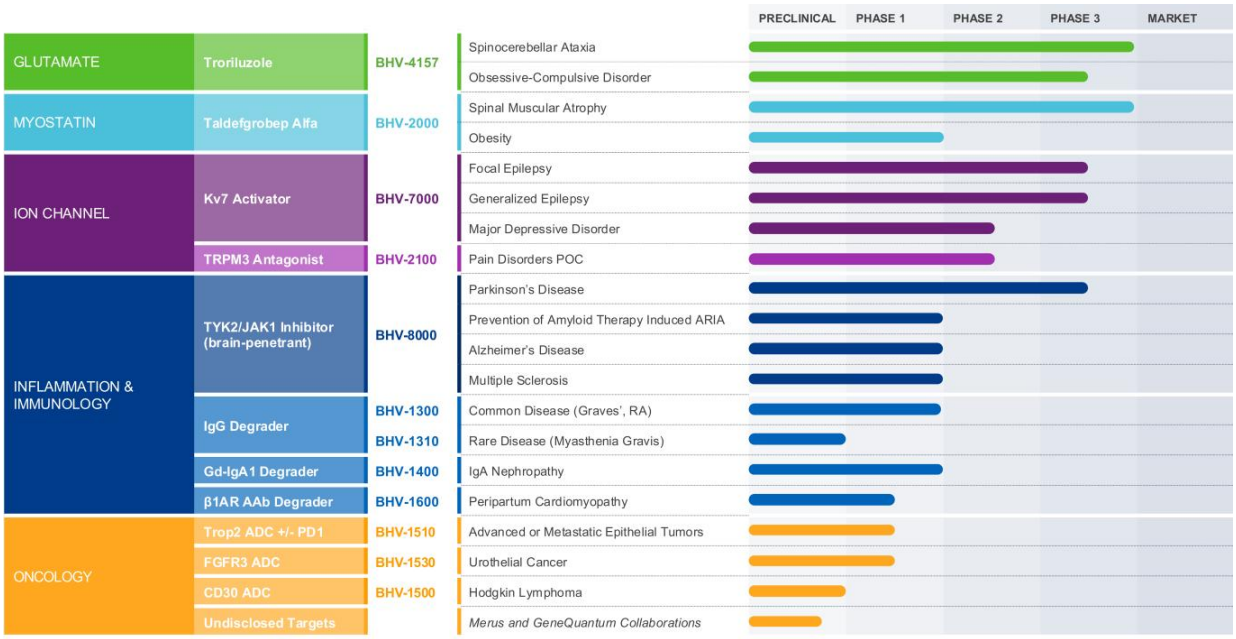
June 2025

Forward-Looking Statement

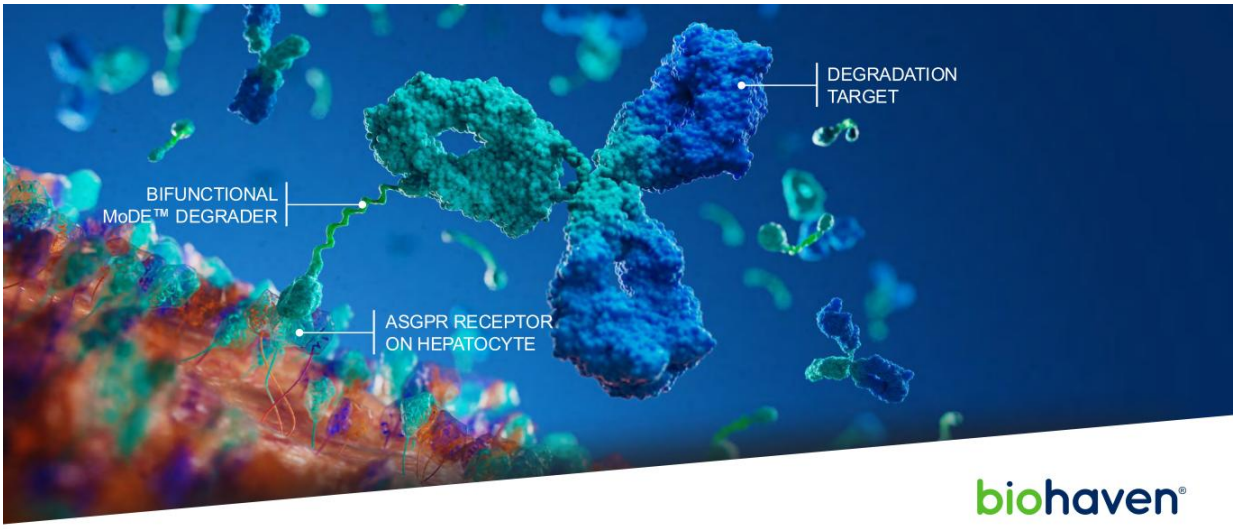
This presentation includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including statements about Biohaven Ltd. (the “Company”) and our planned and ongoing trials for our troriluzole, taldefgrobep alfa, BHV-7000, BHV-2100, BHV-8000, BHV-1300, BHV-1400, BHV-1510, and BHV-1600 development programs, the timing of and the availability of data from our clinical trials, the timing and our decisions to proceed with our planned regulatory filings, the timing of and our ability to obtain regulatory approvals for our product candidates, the clinical potential utility of our product candidates, alone and as compared to other existing potential treatment options, and the potential advancement of our early phase programs including BHV-1310, BHV-1530, and BHV-1500. The use of certain words, including “continue”, “plan”, “will”, “believe”, “may”, “expect”, “anticipate” and similar expressions, is intended to identify forward-looking statements. Investors are cautioned that any forward-looking statements, including statements regarding the future development, timing and potential marketing approval and commercialization of our development candidates, including the potential FDA approval and commercialization of troriluzole for SCA, are not guarantees of future performance or results and involve substantial risks and uncertainties. Actual results, developments and events may differ materially from those in the forward-looking statements as a result of various factors including: the expected timing, commencement and outcomes of Biohaven's planned and ongoing clinical trials; the timing of planned interactions and filings with the Food and Drug Administration, including those regarding the potential FDA approval of troriluzole for SCA; the timing and outcome of expected regulatory filings; complying with applicable U.S. regulatory requirements; the potential commercialization of Biohaven's product candidates; the potential for Biohaven’s product candidates to be first-in-class, best-in-class, best-in-clinic or best-in-category therapies; and the effectiveness and safety of Biohaven's product candidates, including open label clinical data in ongoing studies. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this presentation. Additional important factors to be considered in connection with forward-looking statements are described in the Company’s filings with the Securities and Exchange Commission, including within the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations”. This presentation also contains market data and other information based on industry publications, reports by market research firms or published independent sources. Some market data and information are also based on the Company’s good faith estimates, which are derived from management’s knowledge of its industry and such independent sources referred to above.


DIVERSIFIED PORTFOLIO
TOP AREAS OF INNOVATION
RARE DISEASE
ONCOLOGY
RENAL
CARDIOVASCULAR
OBESITY
NEUROSCIENCE
IMMUNOLOGY & INFLAMMATION
CLINICALLY VALIDATED MoDE™ AND TRAP™ DEGRADERS
ADVANCING CANCER TREATMENTS
Integrated DISCOVERY ENGINE
PIONEERING THERAPIES FOR RARE DISEASES
Working Towards Commercialization of NOVEL THERAPEUTICS
INNOVATING
EXECUTING
CREATING VALUE
biohaven®

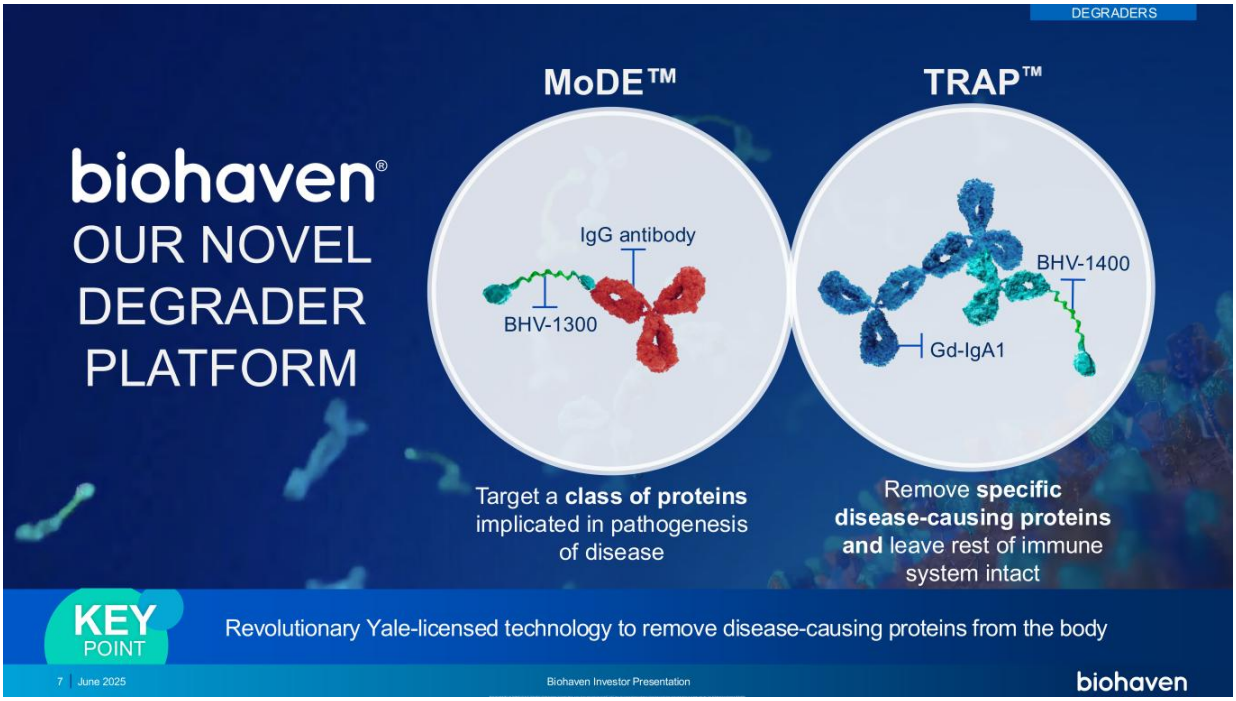

biohaven®
IMMUNOLOGY & INFLAMMATION
Pioneering Degrader Platform With 3 Assets in the Clinic
Initiating Pivotal Trials
Graves' — 2H 2025
IgAN — 1H 2026
Myasthenia gravis — 1H 2026
β1AR — 2H 2026
ONCOLOGY
Proprietary ADC Platform and Collaboration with Merus Bispecifics
2 ADCs in the Clinic
First-in-Clinic FGFR3 ADC
TROP2 Combo with Anti-PD-1
MUSCLE & METABOLISM
Weight Loss
Phase 2 Obesity — 2H 2025
SMA Path Forward
FDA Interaction — 1H 2025
NEUROSCIENCE
Kv7 Program Expecting Pivotal Topline Results
Depression — 2H 2025
Epilepsy — 1H 2026
Neuroinflammation
Initiated Early Parkinson's — 1H 2025
SCA NDA Under Priority Review
PDUFA — 2H 2025
INNOVATING
EXECUTING
CREATING NEAR-TERM VALUE



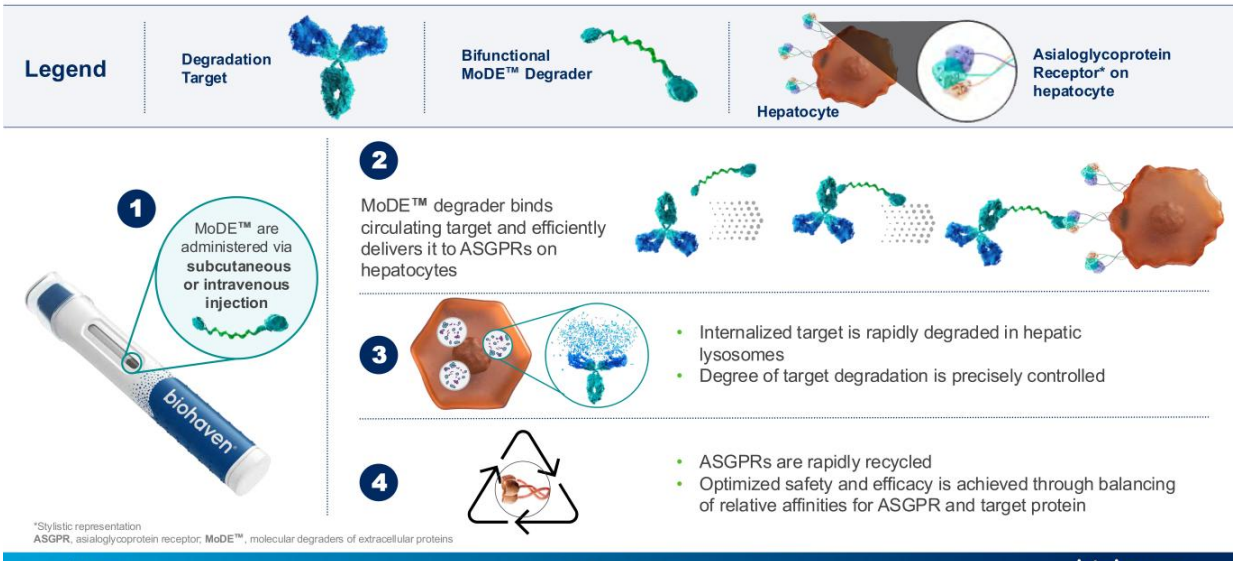
ARIA, Amyloid-related imaging abnormalities; **AAb**, Autoantibody.



MoDE™ and TRAP™ Degraders


DEGRADERS
biohaven®
OUR NOVEL DEGRADER PLATFORM
MoDE™
TRAP™
IgG antibody
BHV-1300
BHV-1400
Gd-IgA1
Target a class of proteins implicated in pathogenesis of disease
Remove specific disease-causing proteins and leave rest of immune system intact
KEY POINT
Revolutionary Yale-licensed technology to remove disease-causing proteins from the body
7 | June 2025
Biohaven Investor Presentation
biohaven

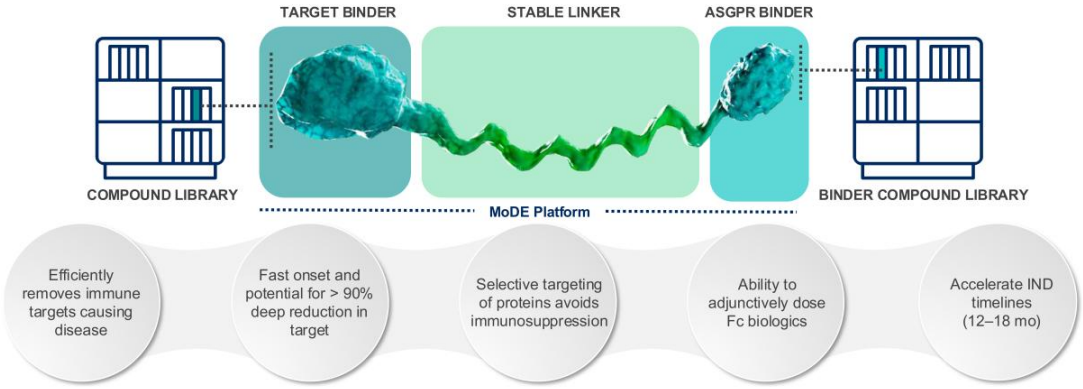
A Novel Mechanism: Hepatic ASGPR Receptor Harnessed for Efficient and Safe Removal of Circulating Pathogenic Targets



*Stylistic representation
ASGPR, asialoglycoprotein receptor; **MoDE™**, molecular degraders of extracellular proteins

A Transformational MoDE™ Drug Platform: Molecular Degraders of Extracellular Proteins (MoDE)

Precisely balanced components selected for optimal efficacy, safety, and product profile



- Efficiently removes immune targets causing disease
- Fast onset and potential for > 90% deep reduction in target
- Selective targeting of proteins avoids immunosuppression
- Ability to adjunctively dose Fc biologics
- Accelerate IND timelines (12–18 mo)

IND, Investigational New Drug Application.

KEY POINT

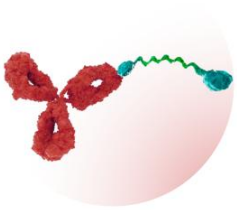
Biohaven's MoDE platform is rapidly generating drug candidates for multiple diseases

Catalyzing Innovation for Patients and Early Demonstration of Removal of Disease-Causing Protein

DEGRADERS

Follow the Science

PRECISION IMMUNOLOGY
TARGET THE DISEASE, NOT THE PATIENT

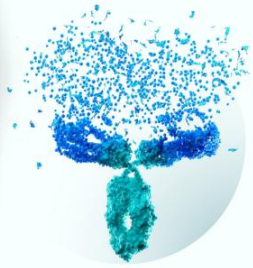


Understand the Need

KEEP THE PATIENT AT THE CENTER

Establish Early Target Efficacy

PHASE 1 PD ENDPOINT

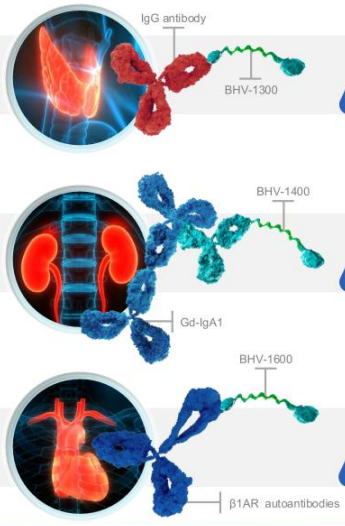


Create Value

NEAR- AND LONG-TERM

INNOVATING | EXECUTING | CREATING VALUE

Biohaven's Novel Degraders Validated in the Clinic: 166 Individuals Dosed With MoDE™ and TRAP™ Degraders



BHV-1300
GRAVES' DISEASE

BHV-1400
IgA NEPHROPATHY

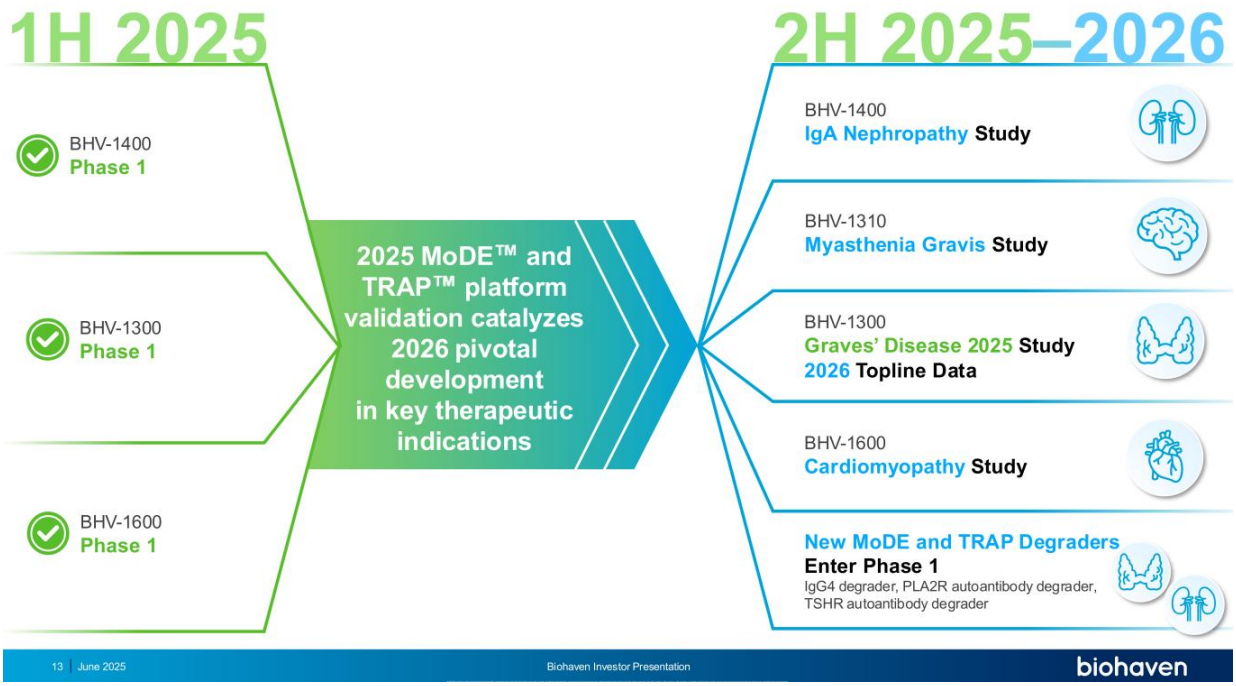
BHV-1600
PERIPARTUM CARDIOMYOPATHY

In ongoing clinical trials

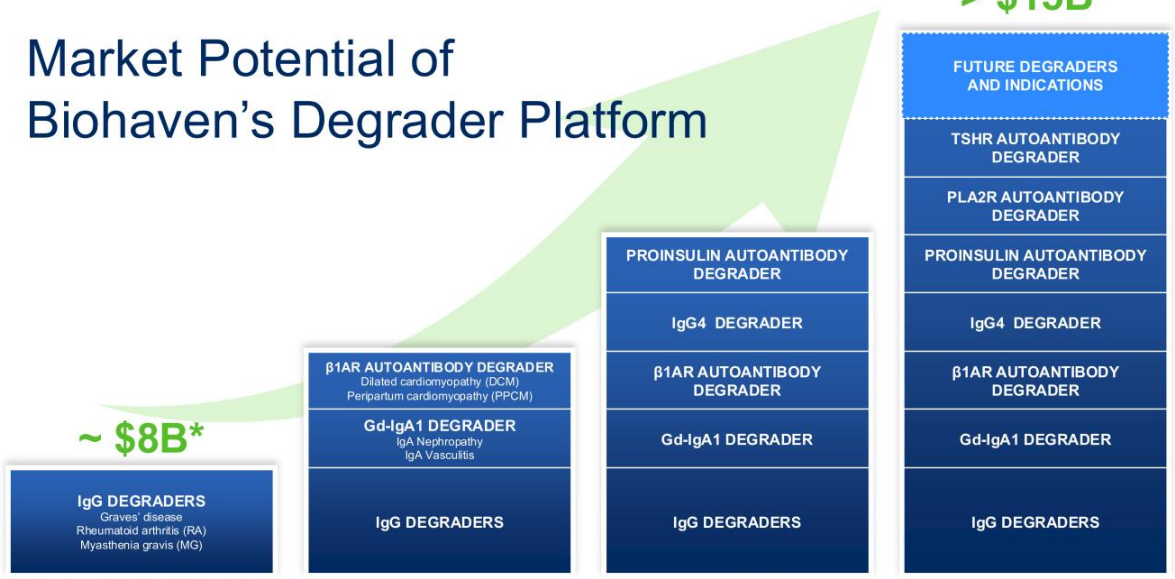
PHASE 1 DATA DEMONSTRATES BIOHAVEN'S MoDE AND TRAP DEGRADERS:

- ✓ Safe
- ✓ Well-tolerated
- ✓ Highly selective
- ✓ Deep and rapid lowering of targeted IgG and Gd-IgA1


DEGRADER
PLATFORM TECHNOLOGY
FAST AND DEEP
Removes disease-causing proteins within hours
SELECTIVE
Designed to target specific pathogenic species for maximal efficacy and minimal side effects
PATIENT CENTRIC
LIFE ALTERING
biohaven
EASY-TO-USE
• Easy-to-use autoinjector for self-administration
• Allows for concomitant use of biologics
TUNABLE
• Level of degradation carefully modulated by dose level and frequency
• Employs body's natural mechanism for removal of senescent proteins
biohaven®


1H 2025
BHV-1400
Phase 1
BHV-1300
Phase 1
BHV-1600
Phase 1
2025 MoDE™ and TRAP™ platform validation catalyzes 2026 pivotal development in key therapeutic indications
2H 2025–2026
BHV-1400
IgA Nephropathy Study
BHV-1310
Myasthenia Gravis Study
BHV-1300
Graves' Disease 2025 Study
2026 Topline Data
BHV-1600
Cardiomyopathy Study
New MoDE and TRAP Degraders
Enter Phase 1
IgG4 degrader, PLA2R autoantibody degrader, TSHR autoantibody degrader

Market Potential of Biohaven's Degrader Platform



* Biohaven Internal Analysis: Peak US Gross Sales


Gd-IgA1 TRAP™ Degrader
biohaven

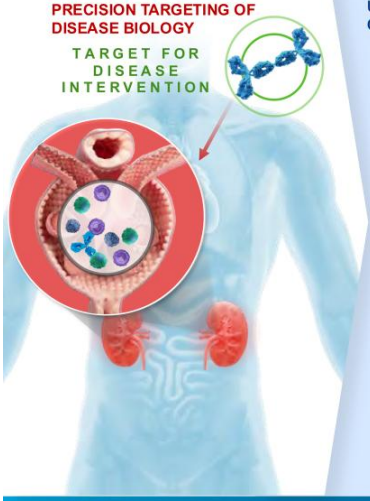
BHV-1400 Selectively Removes Gd-IgA1, the Pathogenic Antibody in IgAN While Sparing Healthy Immunoglobulins

DEGRADERS

Follow the Science

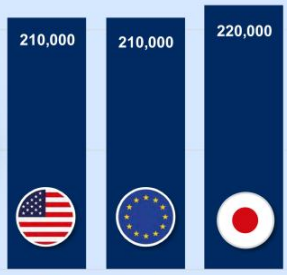
PRECISION TARGETING OF DISEASE BIOLOGY

TARGET FOR DISEASE INTERVENTION



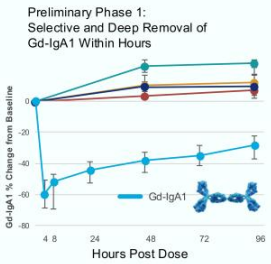
Understand the Need

UNMET NEED & LARGE COMMERCIAL OPPORTUNITY



Demonstrate Early Efficacy

PHASE 1 PD ENDPOINT

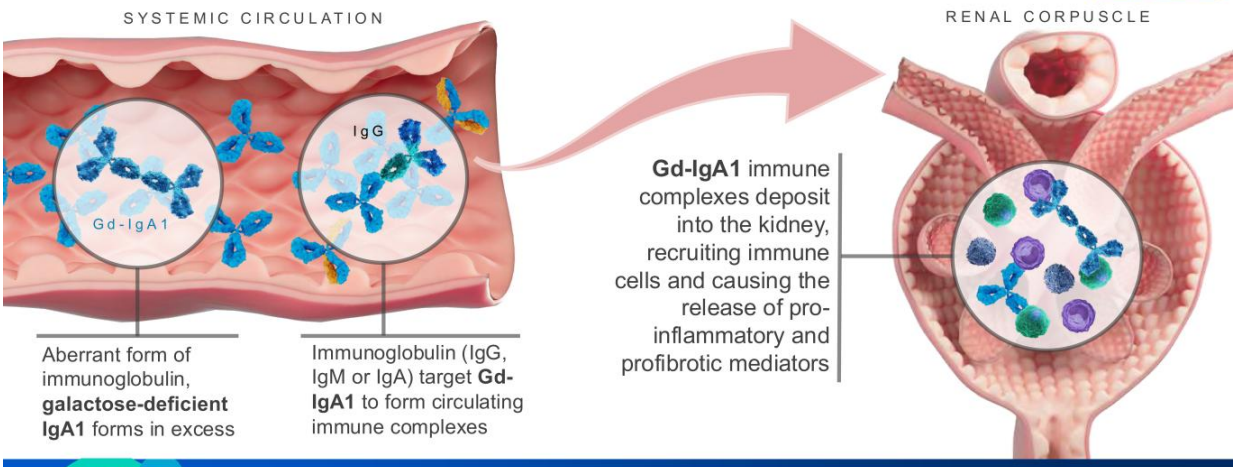


Phase 1 Pharmacodynamic endpoint predicts **success**, enables **speed**; sparing of healthy immune components predicts improved **safety**

Create Value

PIVOTAL TRIAL TO INITIATE 2026

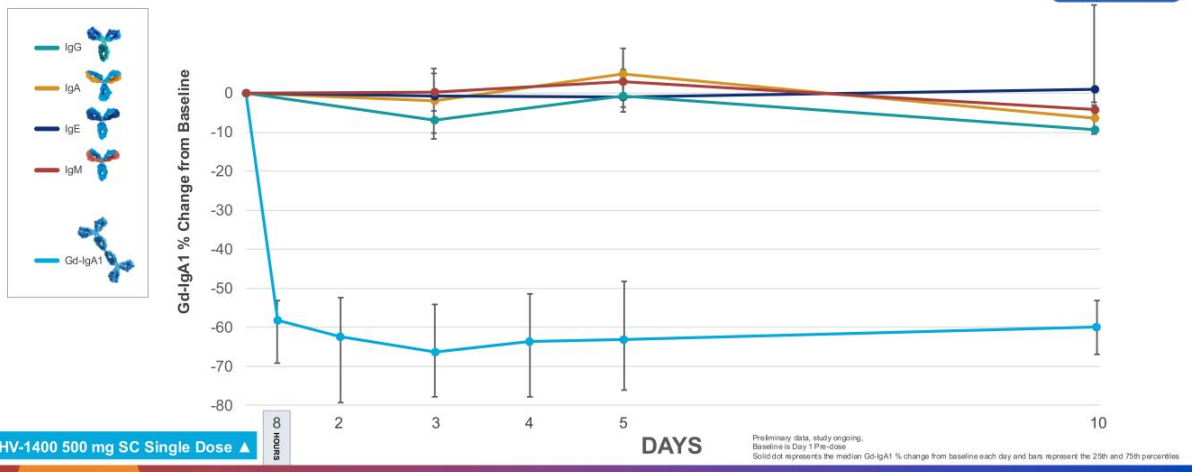
IgA Nephropathy Is Caused by Excess Production of Galactose-Deficient IgA1 (Gd-IgA1)



KEY POINT No therapy selectively targets the pathogenic nidus of disease, Gd-IgA1... **UNTIL NOW**

BHV-1400: Single Subcutaneous Dose Delivers *Rapid, Selective, Deep and Sustained* Removal of Gd-IgA1

DEGRADERS

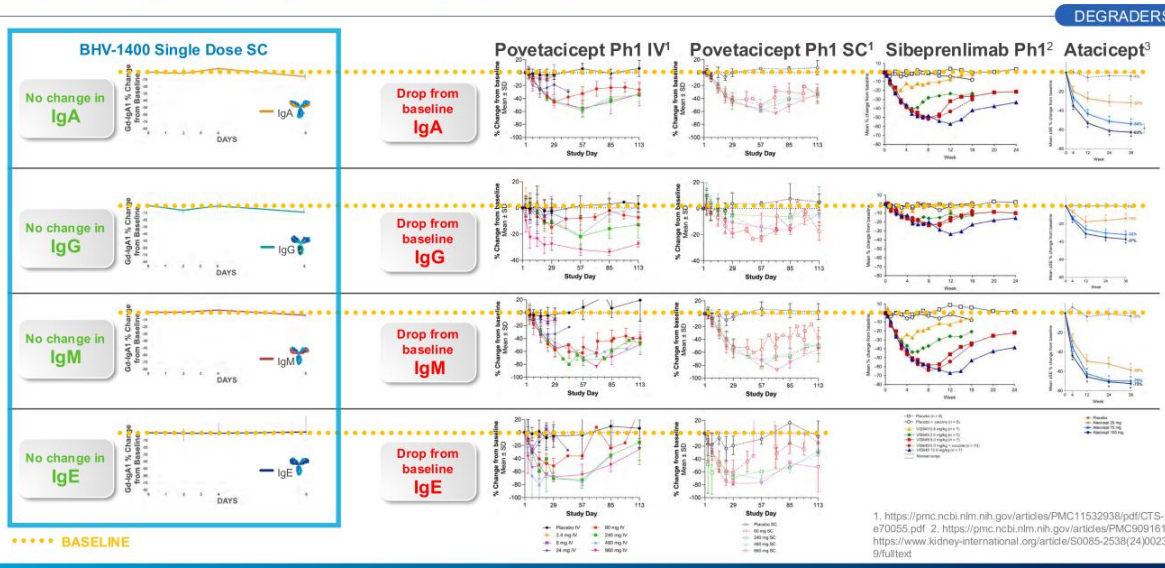


BREAKING NEWS

A single SC dose of BHV-1400 delivers rapid, selective, deep, and sustained reductions in Gd-IgA1 of up to 81% and without suppression of healthy immunoglobulins

BHV-1400 TRAP™ Degrader Targets Gd-IgA1 with Precision Without Reducing Healthy Immunoglobulins

DEGRADERS



1. https://pmc.ncbi.nlm.nih.gov/articles/PMC11532938/pdf/CTS-17-e70055.pdf 2. https://pmc.ncbi.nlm.nih.gov/articles/PMC9091613/ 3. https://www.kidney-international.org/article/S0085-2538(24)00236-9/fulltext

High Selectivity Predicts Improved Safety

DEGRADERS

	BHV-1400	Tarpeyo® (budesonide)	Vanrafia® (astrasentan)	Filspari® (sparsentan)	Fabhalta® (iptacopan)
Risk Evaluation and Mitigation Strategies (REMS)	**Not anticipated**	NO	NO	YES	YES
Black Box	**Not anticipated**	NO	YES embryo-fetal toxicity	YES hepatotoxicity and embryo-fetal toxicity	YES serious infections caused by encapsulated bacteria
Targets	**Selectively targets disease causing protein**	Targets glucocorticoid receptors	Targets endothelin receptor	Targets ETAR and AT1R	Targets complement Factor B

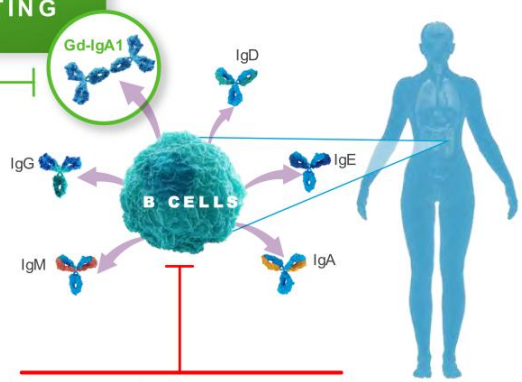
BHV-1400: Selective Removal of Disease-Causing Gd-IgA1 without Immunosuppression Compared to Competitors

DEGRADERS

PRECISION TARGETING

biohaven
TRAP™ Degrader
BHV-1400
SELECTIVELY DEGRADES ONLY Gd-IgA1

Targeting the pathogenesis of disease without immunosuppression



TARGET B CELLS
with global immunoglobulin suppression

Povetacicept — ALPINE IMMUNOSCIENCES / VERTEX
Atacicept — vera therapeutics
Sibeprenlimab — Otsuka
Zigakibart — CHINOOK THERAPEUTICS / NOVARTIS
Felzartamab — Biogen

BROAD IMMUNOSUPPRESSION
TARPEYO® — calliditas

INHIBITS COMPLEMENT SYSTEM
with broad immunosuppression
FABHALTA® — NOVARTIS
RO7434656 — IONIS / Roche
ULTOMIRIS® — ALEXION / AstraZeneca

TARGET ENDOTHELIN RECEPTOR
FILSPARI® — TRAVERE THERAPEUTICS
VANRAFIA® — CHINOOK THERAPEUTICS / NOVARTIS

Harnessing Efficient IgAN Trial Design to Address a High Unmet Need
BHV-1400 Phase 2/3 Study Design

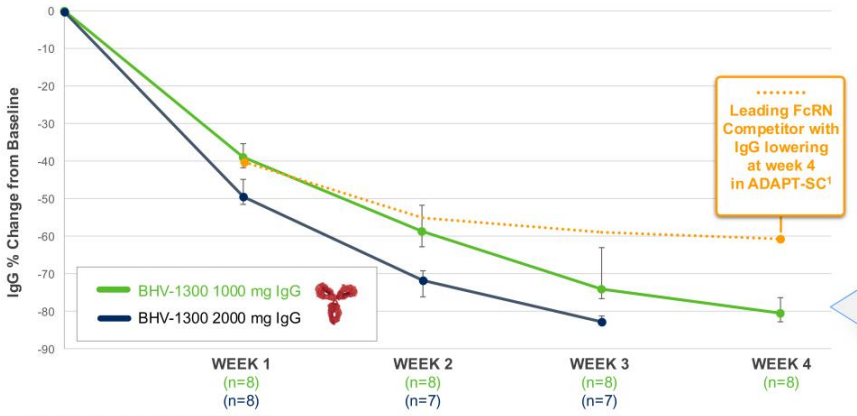
DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Male and female adults with biopsy proven IgA nephropathy
SAMPLE SIZE	500 participants randomized 1:1
TREATMENT	BHV-1400 500 mg SC Q2Wk vs Placebo Q2Wk
TREATMENT DURATION	24-month treatment period
KEY ENDPOINTS	Change in UPCR at 9 months (AA), Annualized total eGFR slope

KEY POINT Accelerated approval pathway to bring a selective, disease-specific therapeutic to treat IgAN


IgG MoDE™ Degrader
biohaven

BHV-1300: Differentiated Small Molecule Degrader Achieves *Deep, Rapid and Tunable* IgG Reductions Customized to the Needs of Specific Diseases

DEGRADERS



Subcutaneous BHV-1300 achieved median maximal reductions in total IgG of

83%

by day 18

1. Adapted from Howard, et al. *Neurotherapeutics*, 2024

Preliminary data, study ongoing, baseline is the Average of Day -1 and Day 1 pre-dose

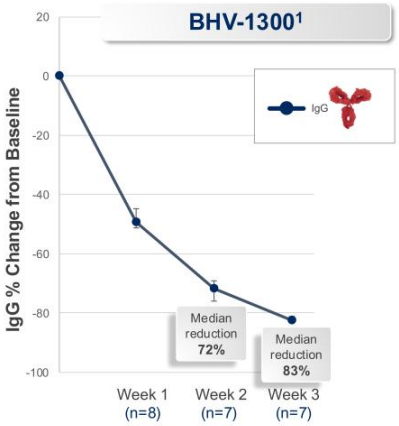
Solid dots represent the median of the maximal total IgG % change from baseline for the Week and bars represent the 25th and 75th percentiles

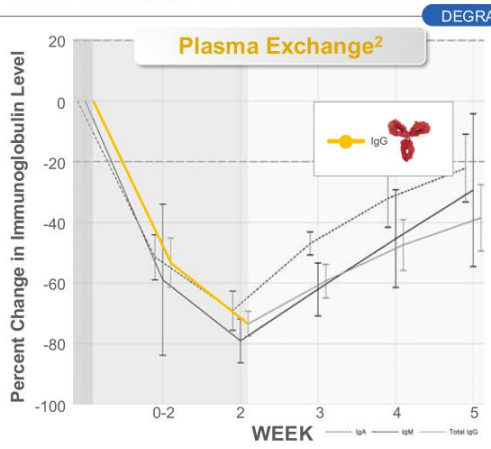
BREAKING NEWS

BHV-1300 achieved rapid and deep IgG reductions of 83%, highlighting tunability of dosing paradigms depending on disease indication

BHV-1300: Offers a New Potential Paradigm for Management of Acute Disease, Lowering IgG as Rapidly and Deeply as Plasma Exchange

DEGRADERS





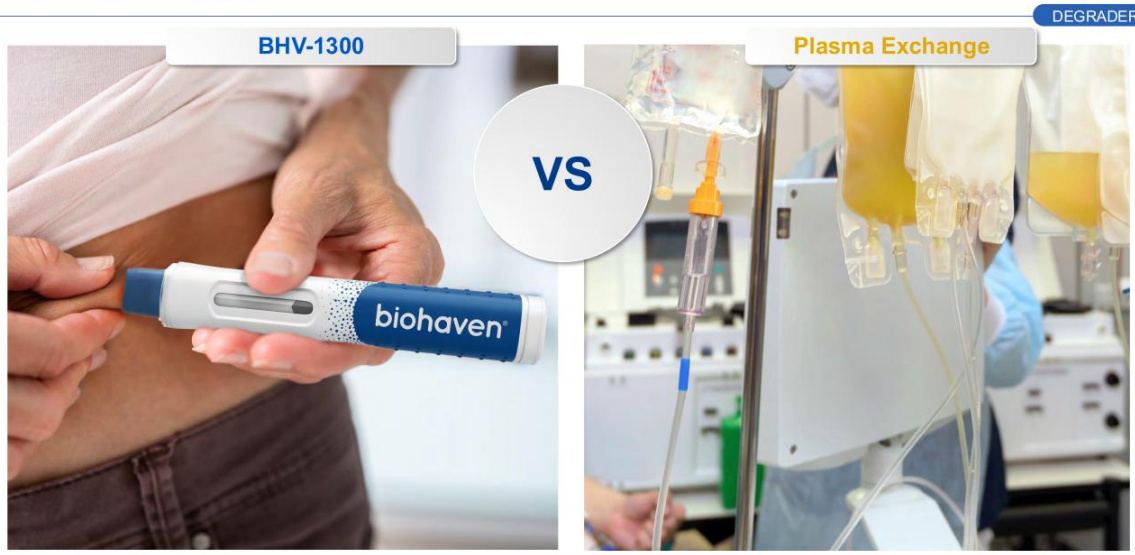
1. Baseline is the Average of Day -1 and Day 1 pre-dose. Solid dot represents the median of the maximal total IgG % change from baseline at each week and bars represent the 25th and 75th percentiles. 2. Guptill JT, et al. *Autoimmunity.* 2016;49(2016):472-9.

KEY POINT

Two doses of BHV-1300 lower IgG as deeply and quickly as these invasive methods including plasma exchange

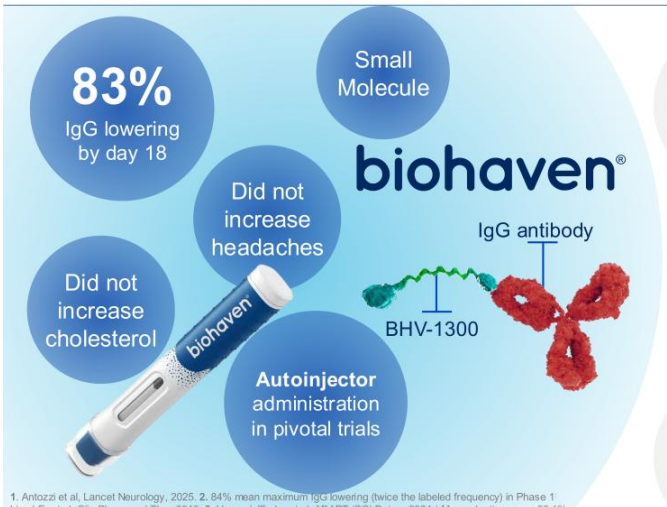
BHV-1300 Could Represent Replacement Plasma Exchange (PLEX) in an Auto-Injector

DEGRADERS



Not Just Another FcRn Inhibitor: Biohaven IgG MoDE™ Degrader Differentiates as a Novel MOA, First-in-Class Molecule

DEGRADERS



IMAAVY™ J&J

- 74.6% IgG lowering after load, 68.8% in maintenance in Vivacity MG-3[1,2]
- **IV infusion**
- Increased cholesterol (24%), muscle spasms (12%), edema (12%)

Vyvgart® argenx

- Approximately 61% IgG lowering @ week 4 (VYVGART Hytrulo® in MG trial)[3] (Average 75% in MAD)[4]
- Prefilled Syringe
- Cyclical dosing can lead to symptom rebound

Rystiggo® UCB

- Approximately 76% IgG lowering in the MycarinG study [5]
- Healthcare administered SC infusion
- **44% headaches**
- Cyclical dosing can lead to symptom rebound

1. Antozzi et al, Lancet Neurology, 2025. 2. 84% mean maximum IgG lowering (twice the labeled frequency) in Phase 1: Ling LE, et al. *Clin Pharmacol Ther.* 2019; 3. Howard JF, Jr., et al. ADAPT (SC) Data – 2024 (Max reduction range 58.1%-63.5%) ; 4. Ulrichts P, et al. *J. Clin. Invest.* 2018 5.Bril et al. *Lancet Neurology*, 2023 – (median maximum IgG reduction 73% with 7mg/kg and 79% with 10mg/kg dose – mean reductions were lower). Rystiggo MAD data not available

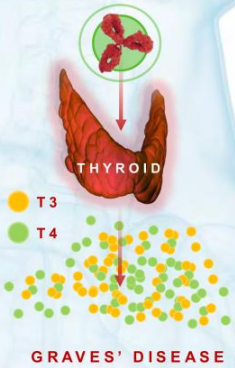
Graves' Disease: Precision Target Selection Enables Speed and Facilitates Confidence of Success

DEGRADERS

Follow the Science

PRECISION TARGETING OF DISEASE BIOLOGY

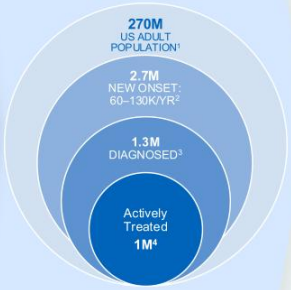
TARGET FOR DISEASE INTERVENTION



GRAVES' DISEASE

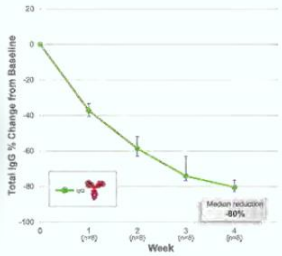
Understand the Need

UNMET NEED & LARGE COMMERCIAL OPPORTUNITY



Demonstrate Early Target Efficacy

PHASE 1 PD ENDPOINT

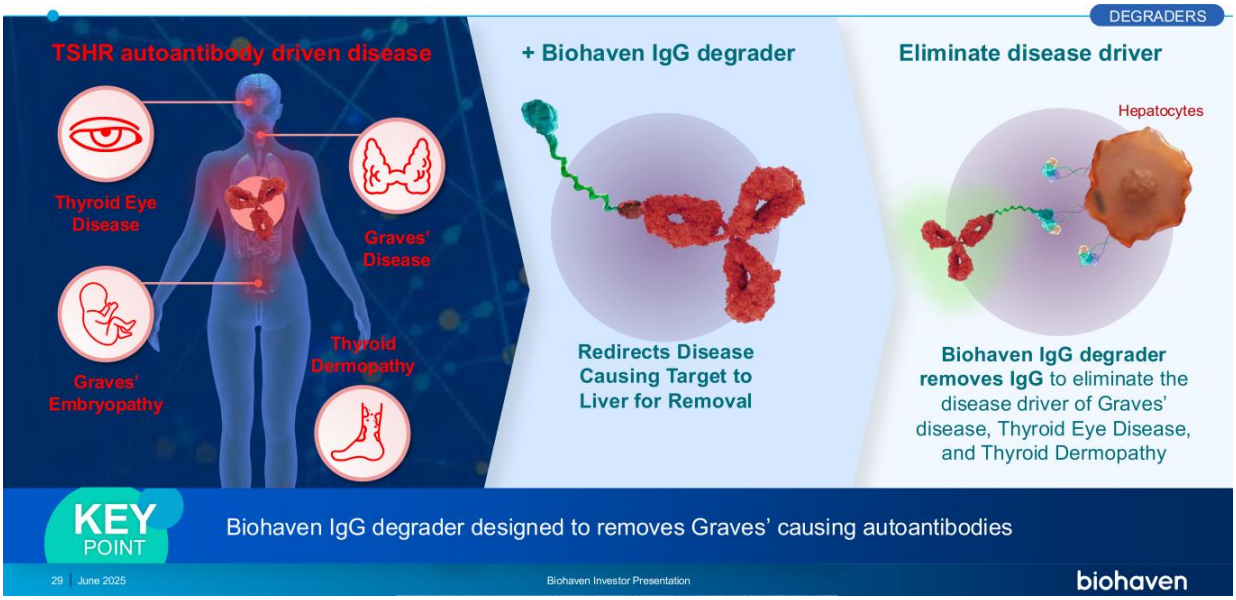


Phase 1 Pharmacodynamic endpoint **predicts success** and **enables speed** in Pivotal Studies

Create Value

PIVOTAL TRIAL TO INITIATE 2H 2025

Biohaven IgG Degrader Targets the Root Cause of a Broad Autoimmune Disease to Treat and Prevent Multi-Organ Complications

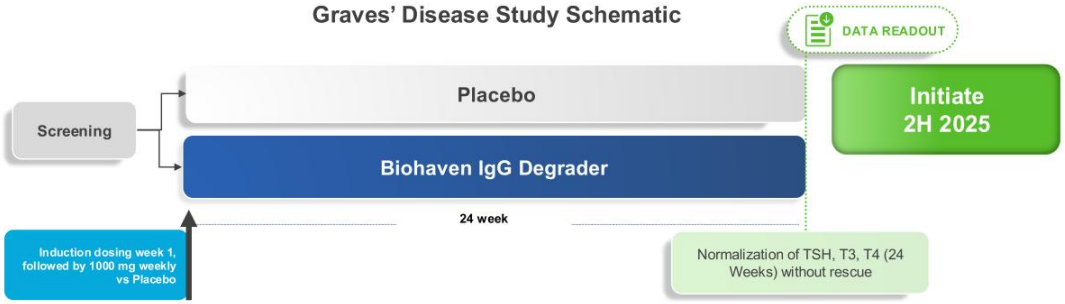


KEY POINT

Biohaven IgG degrader designed to removes Graves' causing autoantibodies

2H 2025 Graves' Disease Pivotal Trial

Graves' Disease Study Schematic



DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Male and female adults with Graves' disease
TREATMENT DURATION	24-week treatment period
KEY ENDPOINTS	Normalization of T3, T4 and TSH without rescue at week 24

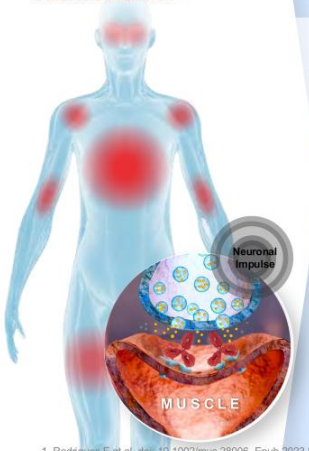
KEY POINT Biomarker driven study enables speed to near-term value inflection

Myasthenia Gravis: Biohaven's IgG Degraders are Tunable Therapy for Acute and Chronic Phases of Disease

DEGRADERS

Follow the Science

PRECISION TARGETING OF DISEASE BIOLOGY



Understand the Need

UNMET NEED & LARGE COMMERCIAL OPPORTUNITY

~100K[1]
US PATIENTS

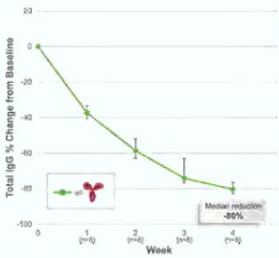
$3.2B
2024 TOTAL
Soliris®
Vyvgart®
Ultomiris®
Rystiggo®
Zilbrysq®

$2.2B
2023 TOTAL
Soliris®
Vyvgart®
Ultomiris®
Rystiggo®

Multi-billion dollar nationally and internationally **growing market opportunity**[2]

Demonstrate Early Target Efficacy

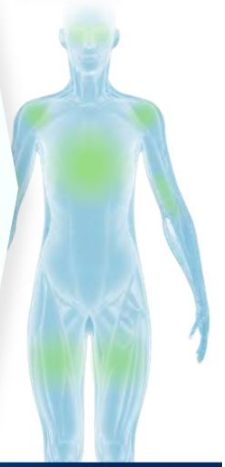
PHASE 1 PD ENDPOINT



Phase 1 Pharmacodynamic endpoint **predicts success** and **enables speed** in Pivotal Studies

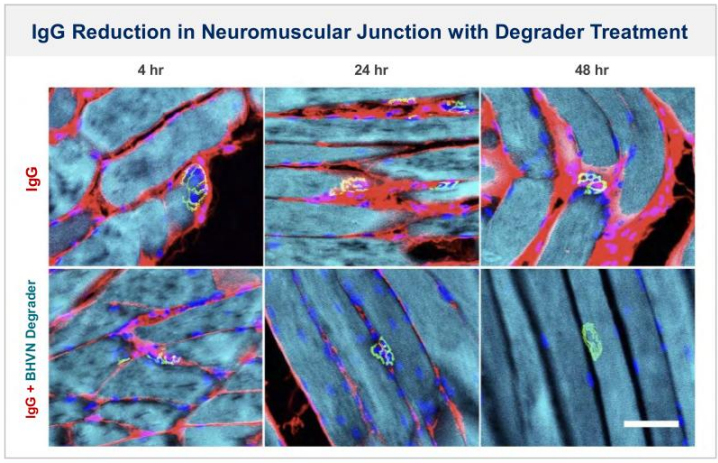
Create Value

PIVOTAL TRIAL TO INITIATE 2026



1. Rodrigues E et al. doi: 10.1002/mus.28006. Epub 2023 Dec 1. PMID: 38040629. 2.Estimated revenue contribution for gMG based on Biohaven internal analysis.

In Vivo Pre-Clinical Validation of the IgG Degrader Mechanism of Action

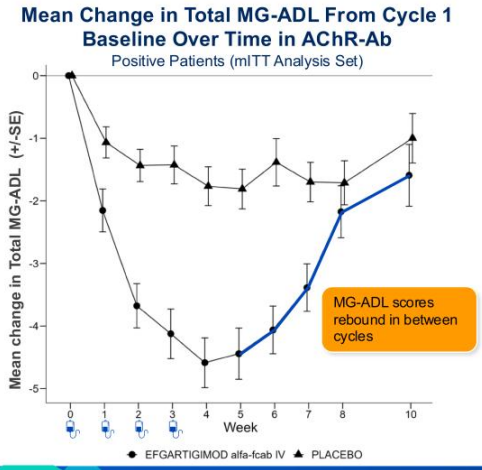


KEY POINT

Biohaven IgG degraders can rapidly clear IgG from the interstitial space and neuromuscular junction

Cycles of Dosing with FcRns Lead to Cycles of Recovery Punctuated by Periods of Symptom Rebound

DEGRADERS



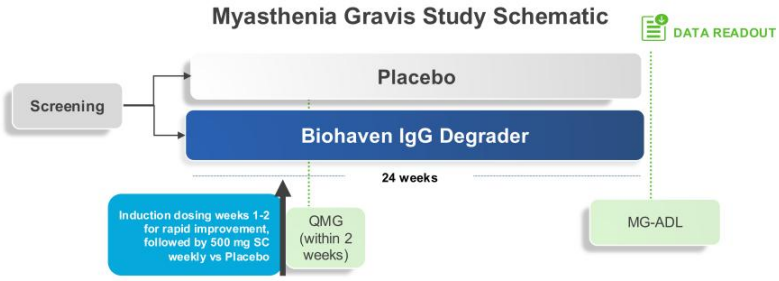
- Chronic diseases like MG require sustained therapy to prevent symptom fluctuations and worsening
- Drug holidays or cyclic dosing can lead to MG-ADL score rebound, disrupting patient quality of life[1,2]

MG-ADL, Myasthenia Gravis Activities of Daily Living scale 1. Vyvgart Hytrulo® Prescribing Information – argenx 2.Rystiggo® Prescribing Information – UCB

KEY POINT

PATIENT-CENTERED CARE: Continuous and sustained dosing (e.g., BHVN degraders) can improve adherence and potentially reduce disease burden

Biohaven IgG Degrader: Clinical Trial Design Highlights Potential Efficacy in Acute and Maintenance MG Therapy



DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Male and female adults with gMG
TREATMENT DURATION	24-week treatment period
KEY ENDPOINTS	QMG (within 2 weeks), MG-ADL (week 24)

QMG, Quantitative Myasthenia Gravis score; **MG-ADL**, Myasthenia Gravis Activities of Daily Living scale

KEY POINT BHV-1300 candidate to treat acute MG exacerbation and maintenance therapy with a single drug

Targeting the Role of Autoimmunity in PPCM

Current treatment for PPCM

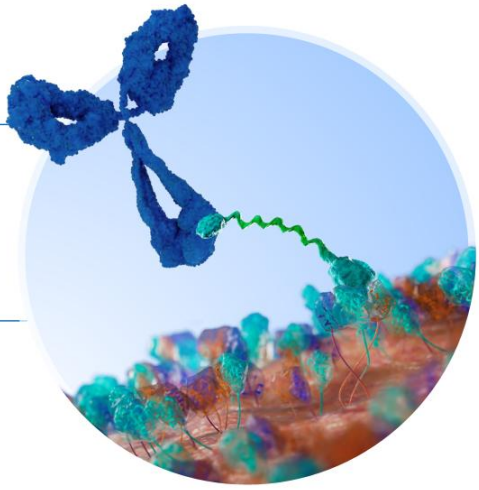
- Guideline-Directed Medical Therapy: No disease specific therapy

Treatment goals in PPCM

- Intervene early
- Treat acute heart failure
- Prevent further myocardial injury and restore baseline normal cardiac function

β1AR AAbs present in PPCM

- Increased titers correlate with more severe disease
- β1AR autoantibodies are a potential target to provide PPCM patients with their first disease specific therapy

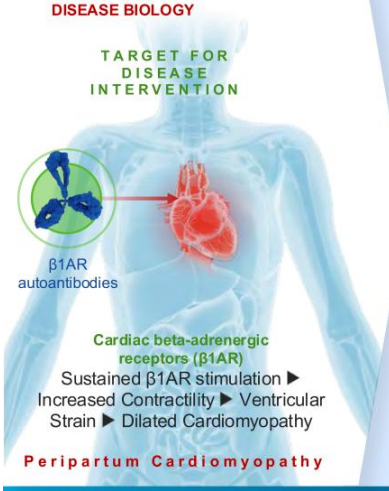


PPCM: BHV-1600 Targets β1AR Autoantibodies for First Potential Disease-Specific Therapy

DEGRADERS

Follow the Science

PRECISION TARGETING OF DISEASE BIOLOGY



Cardiac beta-adrenergic receptors (β1AR)
Sustained β1AR stimulation ▶ Increased Contractility ▶ Ventricular Strain ▶ Dilated Cardiomyopathy

Peripartum Cardiomyopathy

Understand the Need

PPCM is rare disease with poor outcomes in new mothers and no currently FDA-approved treatment

Maternal mortality highest since 1965 and primary contributor is PPCM with mortality rates reported up to 20%

10% go on to require mechanical support (LVAD or heart transplant)

Phase 1 PD Predicts Success in Phase 3

✓ **PHASE 1 SAD/MAD**
Completed dosing, β1AR PD biomarker results pending

Create Value

PHASE 1 SAD/MAD completed dosing: IV and SC dosing up to 500mg IV

2026 STUDY in PPCM patients

BHV-1600: Phase 2/3 Pivotal Study Design Based Upon FDA INTERACT

PPCM Study Schematic

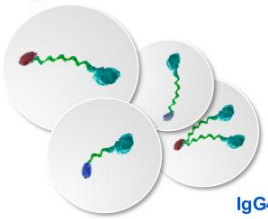
KEY POINT

Pivotal trial plans to leverage accelerated approval pathway to bring a much-needed therapeutic to women with PPCM efficiently

Next-Gen Degraders

Biohaven Discovery Provides Pipeline Sustainability and Optionality

DISCOVERY



DEGRADER PORTFOLIO
MoDEs™, TRAPs™, New Degrader Technology

	preDC	DC*
IgG4: BHV-1450		✓
IgG-Driven Disease: BHV-1320		✓
Anti-PLA2r: Nephropathy		✓
Anti-Proinsulin: Type I Diabetes		✓
Anti-TSHR: Graves' Disease	✓	
Multiple Early Targets	✓	
Technology Build		

*named or planned development candidate within current quarter

SMALL MOLECULES AND BIOLOGIC THERAPEUTICS

preDC	DC*	
	✓	**Kv7.2/3**: Central Backups
	✓	**Kv7**: Peripheral Smooth Muscle
	✓	**TRPM3**: Peripheral and CNS Penetrant
✓	✓	**Neuroscience**: Undisclosed Targets
✓	✓	**Troriluzole**: Backups
✓		**Oncology**: Undisclosed ADC Targets
	✓	**Myostatin Inhibitor**: Backup

KEY POINT

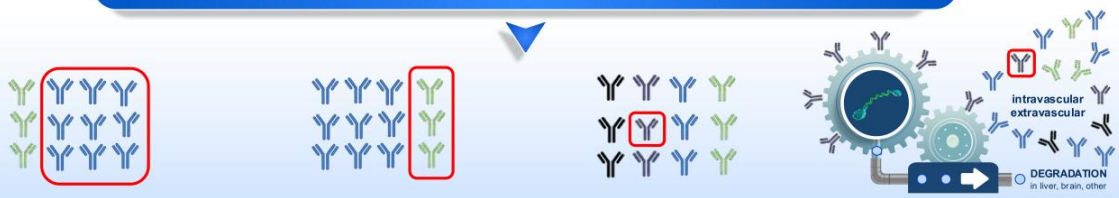
Discovery drug platforms have delivered 6 INDs and multiple drug candidates in 3 years

Expanding the Degrader Platform Into Focused Research Areas

DISCOVERY

- **Neuro and Neuromuscular**: MG, MuSK, CIDP, LRP4, Guillain Barré, MS, MOGAD, NMO*
- **Renal**: IgAN, PLA2r, IgG4, GBM, nephrin-1, cortico-interstitial fibrosis, ADAs*
- **Hematology**: Gammopathies, TTP, wAIHA, CAG, IgA1 and ANCA-associated vasculitis*

Aligning Disease Indications with Appropriate Degrader Technology



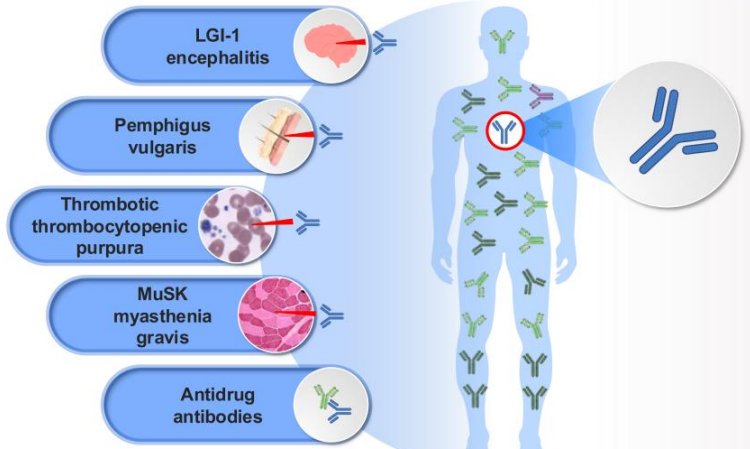
MoDE™ pan-IgG	MoDE IgG Subclass	TRAP™ Targeting AAb	Novel Approaches
e.g., Graves'	e.g., MuSK myasthenia gravis	e.g., Type I Diabetes	e.g., Light chain gammopathy

*Indications are exemplary, many of which are early programs

MG, Myasthenia gravis; **MuSK**, muscle-specific kinase; **CIDP**, chronic Inflammatory demyelinating polyneuropathy; **LRP4**, low-density lipoprotein receptor-related protein 4; **MS**, multiple sclerosis; **MOGAD**, myelin oligodendrocyte glycoprotein antibody disease; **NMO**, neuromyelitis optica; **IgAN**, IgA nephropathy; **PLA2r**, phospholipase A2 receptor; **IgG4**, Immunoglobulin G4; **ADAs**, Autosomal Dominant Alport Syndrome; **TTP**, thrombotic thrombocytopenic purpura; **wAIHA**, warm autoimmune hemolytic anemia; **CAG**, cytarabine, adarubicin, and G-CSF; **IgA1**, immunoglobulin A1; **ANCA**, anti-neutrophil cytoplasmic antibodies.

BHV-1450: Deep Removal of IgG4 for IgG4-Mediated Diseases

DISCOVERY



IgG4-related diseases result in target organ inflammation and fibrosis

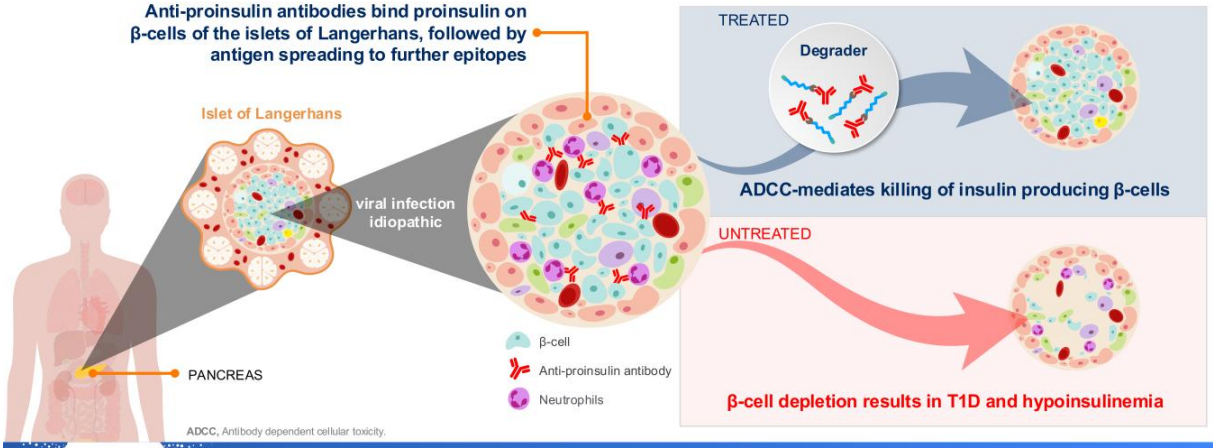
- Pancreatitis
- Sclerosing cholangitis
- Retroperitoneal fibrosis
- Tubulointerstitial nephritis
- Mikulicz's disease and sclerosing sialodacryoadenitis
- Riedel's thyroiditis
- Interstitial lung disease

Mori S et al., *Am J Pathol.* 2012 Feb;180(2):798-810. Koneczny I. *Autoimmun Rev.* 2020;19(10):102646. van Sonderen et al., *Nat Rev Neurol* 13, 290–301 (2017).

KEY POINT

BHV-1450 degrades the minor (4%) IgG4 subclass. IgG1, IgG2 & IgG3 remain unchanged

Removal of Proinsulin Autoantibodies Halts Progression of Nascent Type 1 Diabetes (T1D)

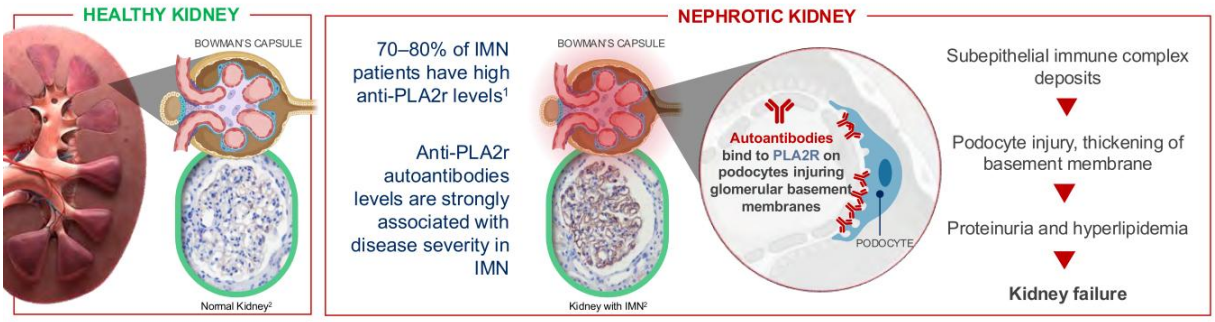


ADCC, Antibody dependent cellular toxicity.

KEY POINTS

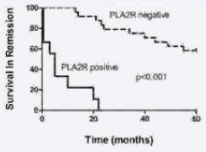
THERAPEUTIC HYPOTHESIS Lowering of antibodies early in course of disease may prevent loss of β-cells and stop cascading events which lead to Type 1 Diabetes

Selective Targeting of Anti-Phospholipase A2 Receptor (PLA2r) Antibodies for Idiopathic Membranous Nephropathy (IMN)



Currently no specific therapies to treat IMN[2]

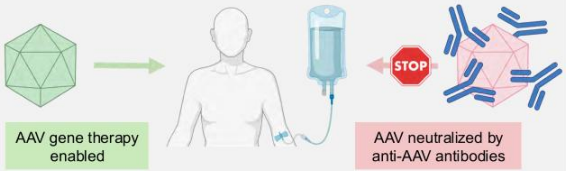
- Rituximab or cyclophosphamide + glucocorticoids are first-line therapies but have serious side effects
- Combination of plasmapheresis with SoC shows more favorable outcomes[3,4]



Patients rendered anti-PLA2r negative by immunosuppression have greater disease remission

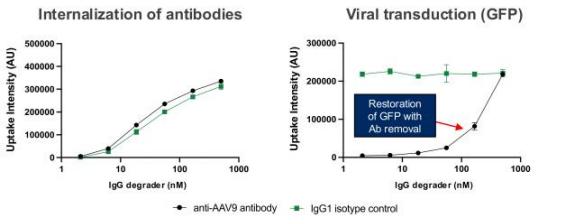
1. Beck, L.H.; Bonegio, R.G.B.; Lambeau, G.; Beck, D.M.; Powell, D.W.; Cummins, T.D.; Klein, J.B.; Salant, D.J. M-Type Phospholipase A 2 Receptor as Target Antigen in Idiopathic Membranous Nephropathy. *N. Engl. J. Med.* 2009, *361*, 11–21. 2. Adapted from *Kidney Intemational* (2012) 82, 797–804 3. Rovin BH, Adler SG, Barratt J, Bridoux F, Burdge KA, Chan TM, et al. KDIGO 2021 Clinical Practice Guideline for the Management of Glomerular Diseases. Kidney Int (2021) 100(4, Supplement):S1–276. doi: 10.1016/j.kint.2021.05.021. 4. Bennani HN, et al.,*J. Pers. Med.* 2024, *14*(3), 249. 5. Lu H et al. Medicine(Baltimore) 2019 May; 98(18): e15303.

Removal of Neutralizing Antibodies to Capsids to Optimize Gene Therapy Uptake and Allow Repeat Administration



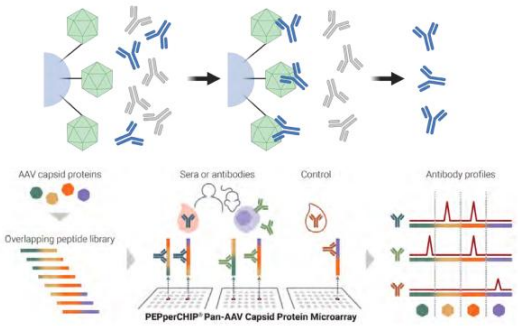
Preexisting anti-AAV antibodies limit the use of AAV-based gene therapy

POC Using GFP Viral Transduction

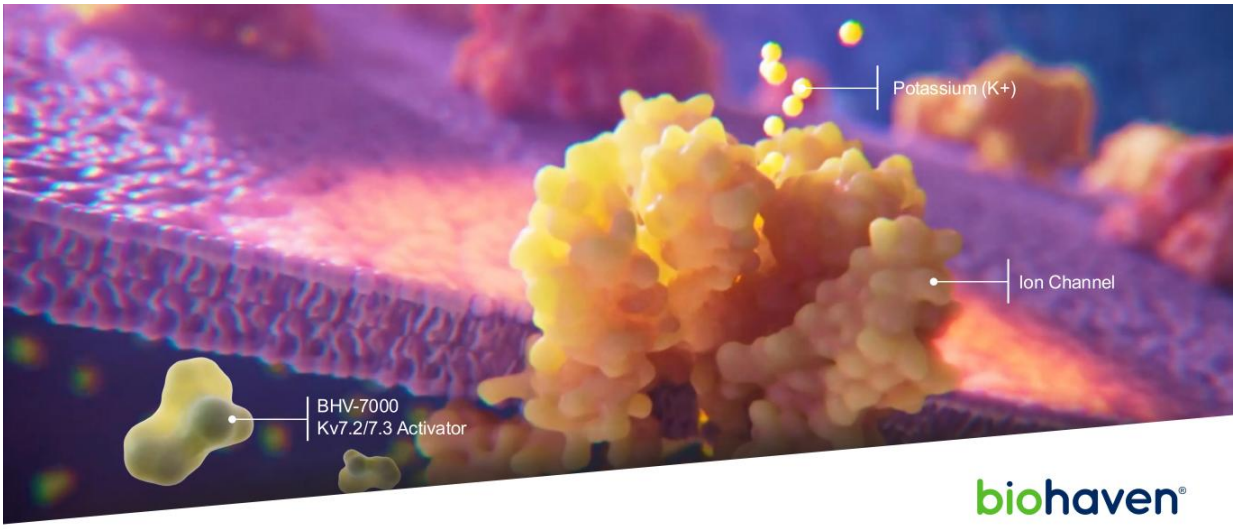


Discovery of Dominant Epitopes for Anti-AAV Antibodies

Enrich anti-AAV antibodies (blue) with immobilized capsids identify binders using array of display techniques



Peptide binders which bind comprehensive antibody populations can be quickly converted to MoDEs



Ion Channel Platform

BHV-7000:
Kv7 Activator for Epilepsy and Depression

Kv7 Is a Transformational Target in Neurology and Neuropsychiatry

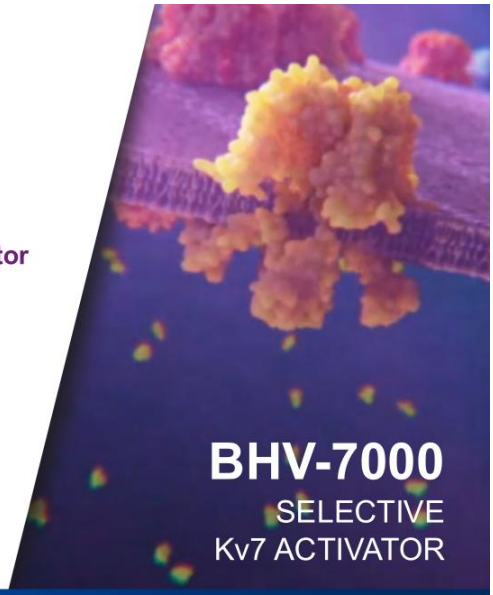
- Selective Kv7 activation avoids unwanted CNS side effects
- Clinically validated in epilepsy, depression and pain

BHV-7000 Is Potential Best-in-Class Selective Kv7 Activator with Blockbuster Potential

- Rationally designed to eliminate $GABA_A$ receptor activation
- No dose-limiting CNS side effects observed in Phase 1
- CNS target engagement at predicted therapeutic concentrations confirmed in Phase 1 EEG study

Late-stage Clinical Development

4 pivotal trials ongoing in epilepsy and MDD



GABA, γ-aminobutyric acid.

KEY POINT

- Pivotal MDD topline results expected 2H 2025
- 1st focal epilepsy study topline results expected 1H 2026

BHV-7000: Potential Best-in-Clinic Selective Kv7 Activator Nears Completion of Pivotal Trials with Blockbuster Potential

Major Depressive Disorder
21M Patients

- Clinically validated MOA for MDD
- Differentiated profile vs. SSRIs

Topline results expected in 2H 2025

Epilepsy
3.5M Patients

- Clinically validated MOA for epilepsy
- Global Phase 2/3 program ongoing in focal epilepsy (2 trials) and idiopathic generalized epilepsy (1 trial)

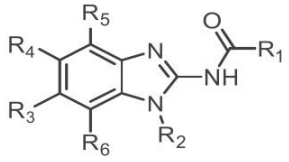
1st focal epilepsy study topline results expected in 1H 2026

BHV-7000 Med-Chem Designed to Fully Leverage Kv7 Target Potential

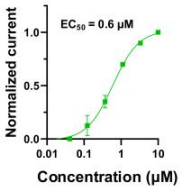
Key areas of differentiation to discover and develop best-in-class Kv7 activator

Pharmacophore-based design principles	Screening and Tier I ADME	Advanced bridging *in vitro* and *in vivo* assays	Clinical
Novel scaffolds	Functional primary screen	Potent context pharmacology	Wide therapeutic index
Photostability	Off-target screening (GABA)	Anti-seizure activity and tolerability	CNS active without delta enhancement

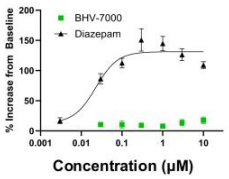
Bicyclic Imidazole



Concentration Dependent Activation

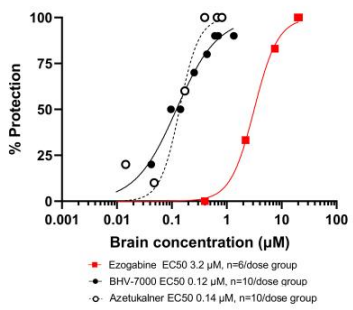


$GABA_A$ PAM activity at α1β3γ2 receptor

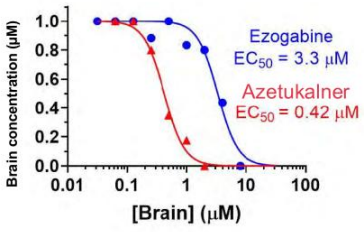


BHV-7000 Equipotent With Azetukalner in Preclinical MES Assay

Efficacy of BHV-7000, Azetukalner and Ezogabine in the Rat AC-MES Assay



Efficacy of Azetukalner and Ezogabine in the Mouse AC-MES Assay[1]

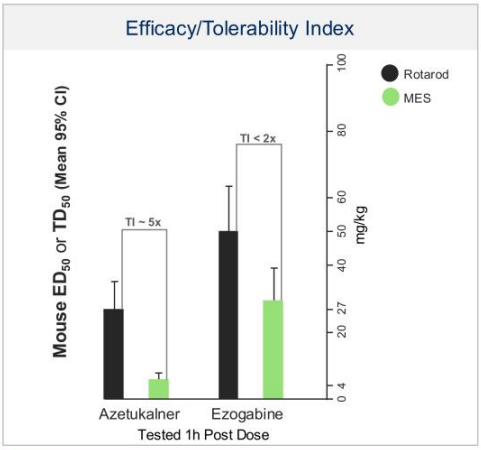


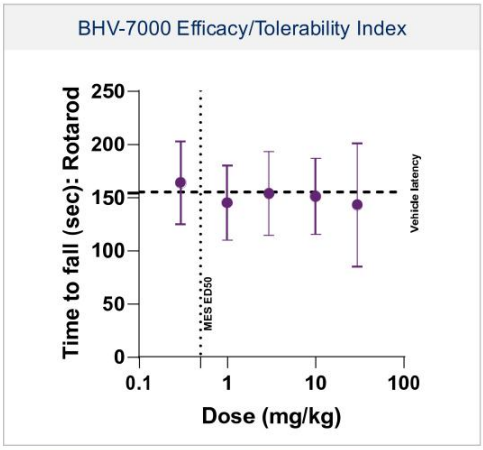
1. Dean, AES 2020. Poster #654.

KEY POINT

BHV-7000 potency consistently ~ 120 nM in complex neuronal system assays

BHV-7000 Differentiated Preclinically From Ezogabine and Azetukalner With Superior Tolerability





KEY POINT

No effect of BHV-7000 on rotarod fall latency at 60x the ED_{50} — TI not calculable

Single Doses of BHV-7000 IR Achieve Multiples Above MES EC_{50} and Are Well-Tolerated in Healthy Volunteers

BHV-7000

Dose (mg)	Cmax (ng/mL)	MES EC_{50} (ng/mL)	Cmax: MES EC_{50}	Nervous System AEs, n (%)[1]		
				Somnolence	Dizziness	Headache
25	253 (12)	186	1.36x	0	0	1 (16.7)
50	664 (31)	186	3.57x	0	0	1 (16.7)
100	877 (42)	186	4.72x	0	0	0

Azetukalner[2,a]

Dose (mg)	Cmax (ng/mL)	MES EC_{50}[3] (ng/mL)	Cmax: MES EC_{50}	Nervous System AEs, n (%)		
				Somnolence	Dizziness	Headache
25	45.8 (14.3)	81	0.57x	2 (33.3)	3 (50.0)	0

1. Awsare B, et al. AES 2023. Poster 3.265. 2. Aycardi. AES 2018. Poster 3.282. 3. Dean. AES 2020. Poster 654. AES 2020.
a. Dosed fasted

Multiple Doses of BHV-7000 ER Exceed MES EC_{50} Over Entire Dosing Interval and Are Well-Tolerated in Healthy Volunteers

BHV-7000

QD x 7 days (mg)	Cmax (ng/mL)	Ctau (ng/mL)	MES EC_{50} (ng/mL)	Cmax: MES EC_{50}	Ctau: MES EC_{50}	Nervous System AEs[1]		
						Somnolence	Dizziness	Headache
50	406 (21.8)	265 (26.0)	186	2.18x	1.42x	0	0	0

Azetukalner[2,a]

QD x 10 days (mg)	Cmax (ng/mL)	Ctau[3] (ng/mL)	MES EC_{50}[4] (ng/mL)	Cmax: MES EC_{50}	Ctau: MES EC_{50}	Nervous System AEs, n (%)		
						Somnolence	Dizziness	Headache
25	96.7 (8.6)	59.5	81	1.19x	0.73x	4 (66.7)	2 (33.3)	3 (50.0)

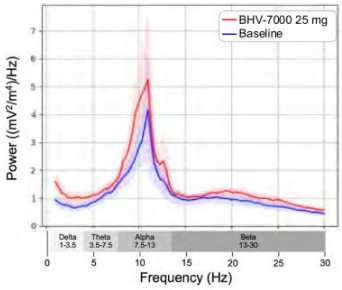
1. Awsare B, et al. AES 2024. Poster 486. 2. Aycardi, AES 2018. Poster 3.282, AES 2018. 3. Digitized estimate from Aycardi, AES 2018. Poster 3.282. 4. Dean, AES 2020. Poster 654.
a. Dosed fed

KEY POINT

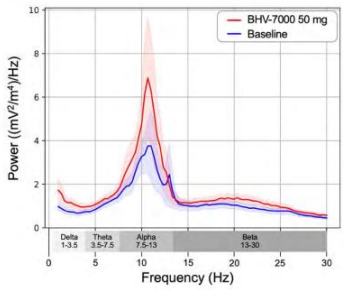
In contrast to BHV-7000, azetukalner exhibits dose-limiting CNS tolerability issues in healthy volunteers at exposures predicted to be effective by preclinical MES data

Dose-Response BHV-7000 CNS Activity Demonstrated in EEG Study

Spectrum at Broadband Max Response
BHV-7000 25 mg vs Baseline[1]



Spectrum at Broadband Max Response
BHV-7000 50 mg vs Baseline[1]



		Nervous System AEs		
Single Dose (mg)	Cmax (ng/mL)	Somnolence	Dizziness	Headache
25	309 (35.7)	0	0	0
50	537 (36.4)	0	0	0

1. Lerner J, et al. AES 2023. Poster 2.510.

Excellent CNS Tolerability Observed in Ongoing Phase 2/3 Focal Epilepsy Studies (Blinded Data) Consistent With Phase 1 Profile

BHV-7000

	Nervous System AEs, %		
Doses (mg)	Somnolence	Dizziness	Headache
25/50/75	1.7	1.1	3.3

No AEs observed at > 5% in any SOC; studies are still ongoing

Azetukalner[1]

	Nervous System AEs, %		
Doses (mg)	Somnolence	Dizziness	Headache
10/20/25	15.6	24.6	10.0

1. French JA, et al. *JAMA Neurol.* 2023;80(11):1145-1154.

KEY POINT

BHV-7000 well-tolerated in focal epilepsy at exposures predicted to be effective based on preclinical data and associated with CNS activity on EEG in healthy volunteers

BHV-7000: Two Phase 2/3 Studies in Focal Epilepsy Ongoing

DESIGN	Randomized, double-blind, placebo-controlled trials
POPULATION	Subjects 18-75 years of age with refractory focal epilepsy
SAMPLE SIZE	390 subjects in each study (randomized 1:1:1)
KEY ENTRY CRITERIA	Average of ≥4 observable focal seizures per 28 days
ENDPOINTS	Change in seizure frequency, 50% responder rate, seizure freedom

BHV-7000: Phase 2/3 Study in Idiopathic Generalized Epilepsy Ongoing

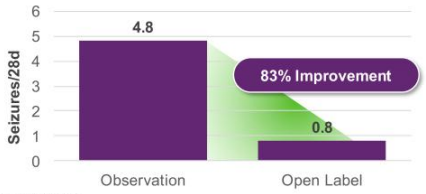
DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects 18-75 years old with idiopathic generalized epilepsy with intractable generalized tonic-clonic seizures
SAMPLE SIZE	242 subjects (randomized 1:1)
TREATMENT	BHV-7000 75 mg vs. placebo
TREATMENT DURATION	Up to 24-week double-blind phase
ENDPOINT	Time to event (2nd day with generalize tonic-clonic seizure)

KEY POINT Pivotal Phase 2/3 IGE study initiated in 1H 2024

BHV-7000: Patient Case Report From OLE

Sample Patient Case Report

- **Subject**: 71-year-old male
- **Type of seizure**: focal impaired awareness
- **Seizure description**: tingling and twitching of cheek spreading to neck and arm followed by arm rigidity and impaired awareness
- **Failed treatments**: levetiracetam, valproate
- **Seizure frequency**: 83% improvement from observation



Preliminary data, study ongoing.

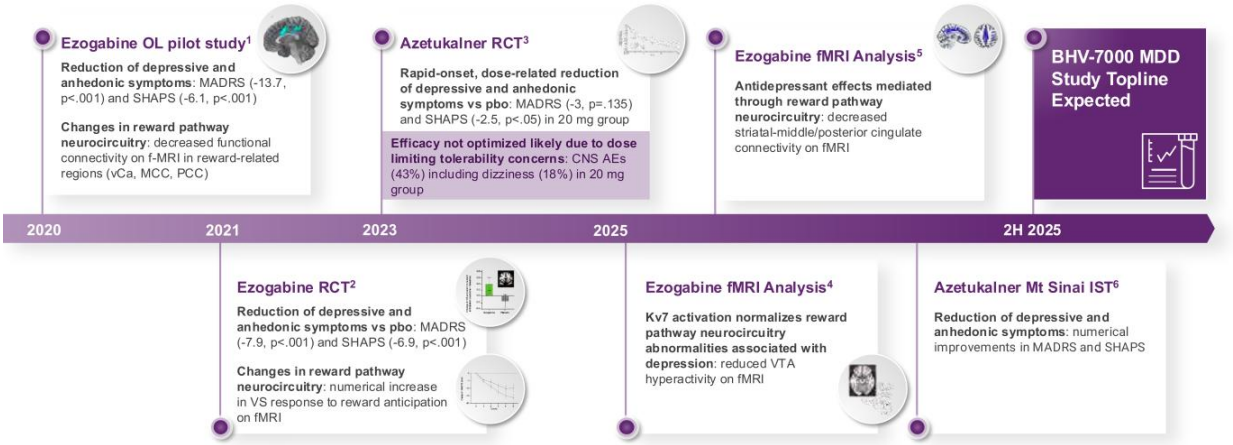
*"Subject has experienced a total of 5 seizures during 176 days in the OLE period. However, he has been **seizure-free for the past 3 months**. Additionally, **no side effects** have been reported during this time. The patient has also demonstrated **meaningful improvement in his quality of life**, reported by both him and his family. They have expressed satisfaction with the treatment and are very positive about their overall experience."*

-- BHV-7000 Focal Epilepsy Clinical Trial Investigator

KEY POINT

BHV-7000 has substantial seizure reduction in selected OLE patient

Increasing Clinical Evidence Supports Potential of Kv7 Activation in MDD



SHAPS, Snaith-Hamilton Pleasure Scale; **MADRS**, Montgomery Åsberg Depression Rating Scale; **OL**, Open-Label; **RCT**, Randomized Control Trial; **fMRI**, Functional Magnetic Resonance Imaging; **IST**, Investigator Sponsored Trial.

1. Tan, et al. *Mol Psychiatry*. 2020 Jun;25(6):1323-1333; 2. Costi, et al. *Am J Psychiatry*. 2021 May 1;178(5):437-446; 3. Butterfield N, et al. ASCP 2024. Poster W67; 4. Morris, et al. *Mol Psychiatry*. 2025 Mar 25; 5. Chowdhury, et al. *Biol Psychiatry*. 2025 Mar 4:S0006-3223(25)01011-X. 6. Xenon Press Release May 12, 2025.

BHV-7000: Phase 2 Study in Major Depressive Disorder Ongoing

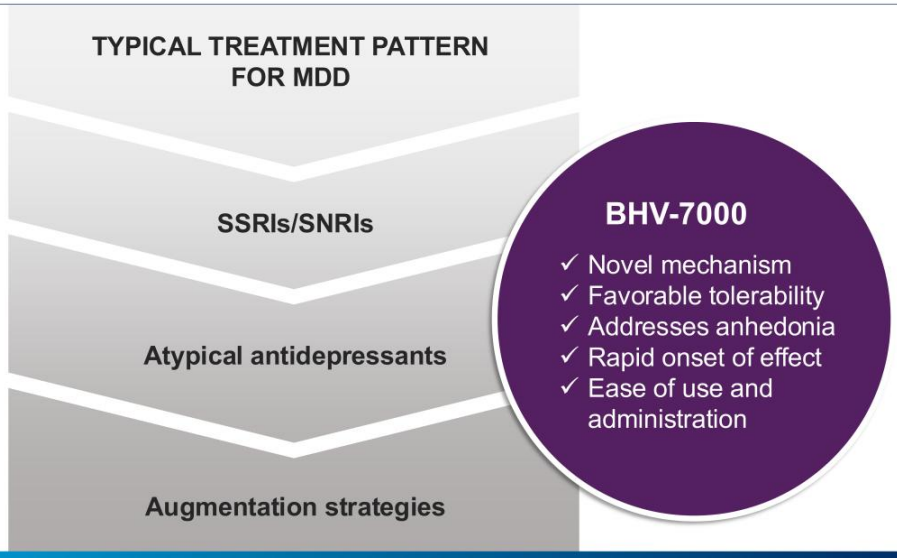
DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Subjects with Major Depressive Disorder (MDD) with at least one prior depressive episode who are currently experiencing a depressive episode with anhedonia (HAM-D ≥ 20, SHAPS ≥ 20)
SAMPLE SIZE	300 subjects (randomized 1:1)
TREATMENT	BHV-7000 vs placebo
TREATMENT DURATION	6-weeks
ENDPOINTS	MADRS (primary), SHAPS, CGI-S, Q-LES-Q-SF

SHAPS, Snaith-Hamilton Pleasure Scale; **MADRS**, Montgomery Åsberg Depression Rating Scale; **CGI-S**, Clinical Global Impression Scale – Severity; **Q-LES-Q-SF**, Quality of Life Enjoyment and Satisfaction Questionnaire.

KEY POINT

Topline results expected 2H 2025

BHV-7000 Has Potential to Overcome Limitations of Existing Therapies



BHV-2100:
TRPM3 Antagonist for Pain

First-in-Clinic TRPM3 Antagonist — Novel Mechanism for the Treatment of Pain

- BHV-2100 is an orally administered, peripherally-restricted and selective TRPM3 antagonist
- Preclinical data shows robust efficacy in pain models
- Highly differentiated from programs that target TRPV1 and TRPA1, avoids TRP family liabilities

Phase 1 Study Data Supports Evaluation in Pain

- BHV-2100 demonstrated excellent safety/tolerability and favorable PK
- Proof of concept demonstrated for pain

The Therapeutic Landscape of TRPM3-Targeted Therapies Is Evolving

- Emerging data support the potential for targeting TRPM3 in nociceptive pain conditions
- Non-clinical data support brain-penetrant TRPM3 program in epilepsy and pain

Milestones Achieved

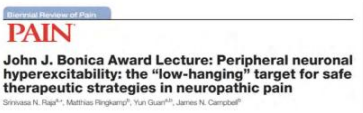
- Early signal of efficacy observed in laser-evoked pain trial
- Proof-of-concept trial in acute treatment of migraine completed with no efficacy signal detected

KEY POINT

Proof-of-concept for pain demonstrated; program advancing to later stage pain trials

BHV-2100: Targeting the Unmet Medical Need in Pain

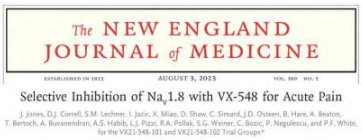
Emerging role of novel mechanisms: ion channels in the periphery


Biennial Review of Pain

PAIN

John J. Bonica Award Lecture: Peripheral neuronal hyperexcitability: the "low-hanging" target for safe therapeutic strategies in neuropathic pain

Srinivasa N. Raja[a,*], Matthias Ringkamp[b], Yun Guan[a,b], James N. Campbell[b]


The NEW ENGLAND JOURNAL of MEDICINE

ESTABLISHED IN 1812 AUGUST 3, 2023 VOL. 389 NO. 5

Selective Inhibition of $Na_v1.8$ with VX-548 for Acute Pain

J. Jones, D.J. Correll, S.M. Lechner, I. Jazic, X. Miao, D. Shaw, C. Simard, J.D. Osteen, B. Hare, A. Beaton, T. Bertoch, A. Buvanendran, A.S. Habib, L.J. Pizzi, R.A. Pollak, S.G. Weiner, C. Bozic, P. Negulescu, and P.F. White, for the VX21-548-101 and VX21-548-102 Trial Groups*

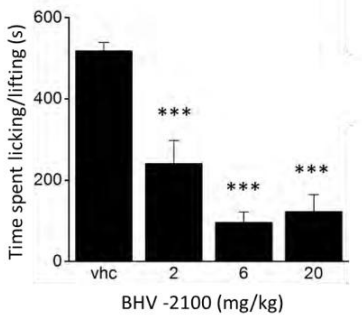
ACUTE PAIN & NEUROPATHIC PAIN

KEY POINT

BHV-2100 is a selective, peripherally-restricted TRPM3 antagonist that is a potentially highly-effective, non-sedating, non-opioid treatment for pain

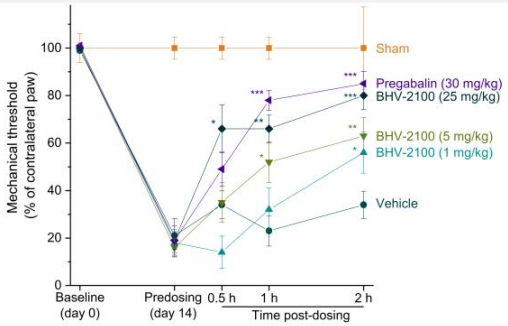
BHV-2100: Potently Reduces Acute Chemogenic Pain and Pain Following Nerve Injury

PS-Induced Acute Pain Model



Drug administered 30 minutes prior to TRPM3 agonist injection in a hind paw of rats

Partial Sciatic Nerve Ligation Model



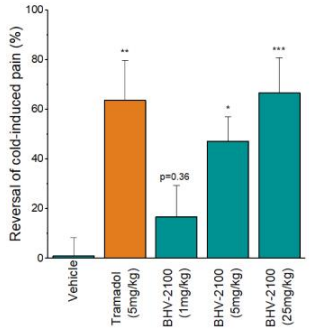
Drug administered 14 days after unilateral sciatic nerve injury in rats

Encouraging evidence of pain reduction without the sedation observed with high-dose pregabalin

*** p<0.001, ** p<0.01 , * p<0.05

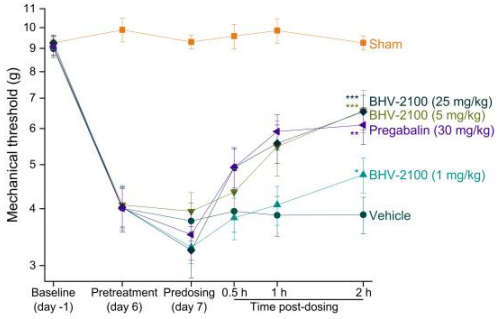
BHV-2100: Reverses Established Pain States in Peripheral Neuropathic Pain Models

Chemotherapy-Induced Neuropathic Pain Model



Drug administered 6 days after oxaliplatin treatment in mice

Diabetic Neuropathy Model



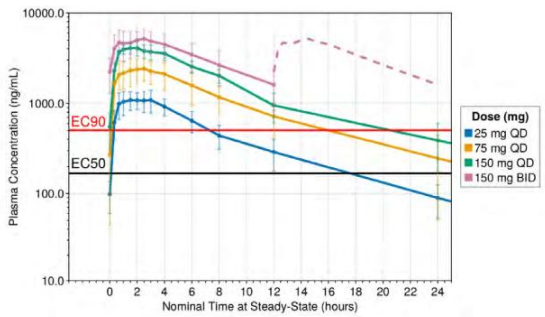
Drug administered 7 days after STZ treatment in rats

Encouraging evidence of pain reduction without the sedation observed with high-dose pregabalin/tramadol

*** p<0.001, ** p<0.01 , * p<0.05

BHV-2100: Ideal Pharmacokinetic Profile for Treating Pain

Mean Concentration vs. Time Profiles at Steady-State
After Multiple Oral Doses of BHV-2100



EC90 represents the estimated plasma concentration threshold based on a preclinical model

KEY POINT

Phase 1 data in healthy subjects shows plasma PK exceeds EC90 by 20 min at all doses tested and is sustained above EC50 for 24 hours at doses > 25 mg QD

BHV-2100: Safe and Well-Tolerated in Healthy Subjects

SAFETY AND TOLERABILITY

- No dose limiting toxicities in studies
- No SAEs
- No severe TEAEs; most TEAEs were mild
- No clinically significant trends in vital signs (including body temperature), laboratory values, or ECGs

DOSING

- SAD: single doses up to 500 mg
- MAD: multiple doses up to 150 mg twice a day for 14 days

SAD Cohorts (pooled) TEAEs in ≥ 2 subjects	Placebo (N=9) n (%)	BHV-2100 (N=30) n (%)
Dizziness	0 (0)	2 (6.7)
Fatigue	0 (0)	2 (6.7)

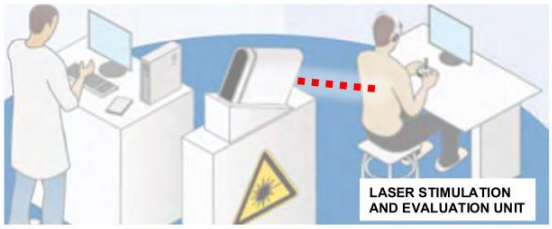
MAD Cohorts (pooled) TEAEs in ≥ 2 subjects	Placebo (N=8) n (%)	BHV-2100 (N=24) n (%)
	0 (0)	0 (0)

MAD, multiple ascending dose; **SAD**, single ascending dose; **SAE**, serious adverse events; **TEAE**, treatment emergent adverse events.
Pooled preliminary data.

KEY POINT

No TEAE occurred in > 1 participant across the MAD cohorts

BHV-2100: Proof of Concept Pain Study Demonstrates Anti-Nociceptive and Anti-Hyperalgesic Effects

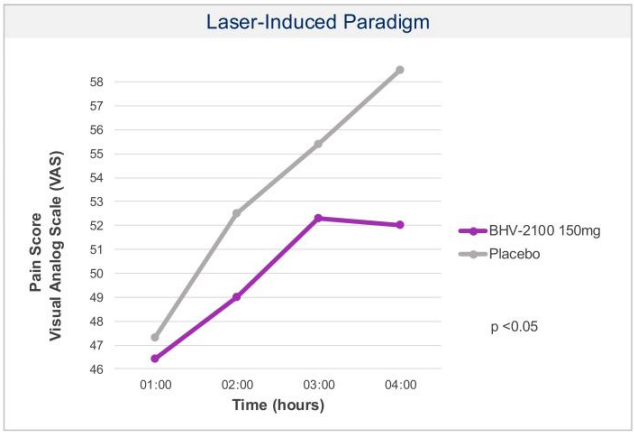


Efficacy

- Lowering in self-reported VAS pain rating scale
- Clinically meaningful reductions in laser-evoked potentials in normal and UVB-inflamed skin

Safety

- Well-tolerated
- No effects observed on core temperature
- No change on heat pain threshold



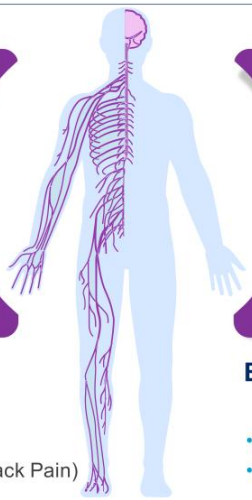
Preliminary Data up to Tmax; p-value out to 8 hour test period

KEY POINT First indication of potential clinical efficacy in pain with the novel TRPM3 mechanism

TRPM3 Platform Offers Potential to Treat an Expanding Range of Diseases

PERIPHERAL NERVOUS SYSTEM
Pain Disorders

- ✓ Mechanistic Data
- ✓ Human Genetic Validation
- ✓ Safety and Tolerability
- ✓ Human Proof of Concept



CENTRAL NERVOUS SYSTEM
Pain and Other Brain Disorders

- ✓ Mechanistic Data
- ✓ Preclinical Proof of Concept

BHV-2100

- Chronic Neuropathic Pain
- Nociceptive Pain (Osteoarthritis)
- Mixed Neuropathic and Nociceptive Pain (Back Pain)
- Acute Pain

Brain-Penetrant TRPM3 antagonists (preclinical)

- Acute and Chronic Pain Disorders
- Epilepsy

biohaven®

BHV-8000:
Brain-Penetrant TYK2/JAK1 Inhibitor for Parkinson's Disease

First-in-Clinic, Brain-Penetrant, Selective TYK2/JAK1 Inhibitor for the Treatment of Neuroinflammatory and Neurodegenerative Diseases

- TYK2 and JAK1 inhibition target key inflammatory signaling pathways
- Selectivity profile avoids safety liabilities of JAK2/3 inhibition

Breaks Cycle of Central and Peripheral Inflammation

- Autoimmunity plays key role in Parkinson's and is novel therapeutic target
- TYK2/JAK1 inhibition reduces inflammatory impact of activated microglia and astrocytes in the CNS, and infiltrating T lymphocytes

Encouraging Results from Phase 1

- Well-tolerated
- Achieves target engagement
- Robust brain penetration

Phase 2/3 Parkinson's Disease Study Ongoing

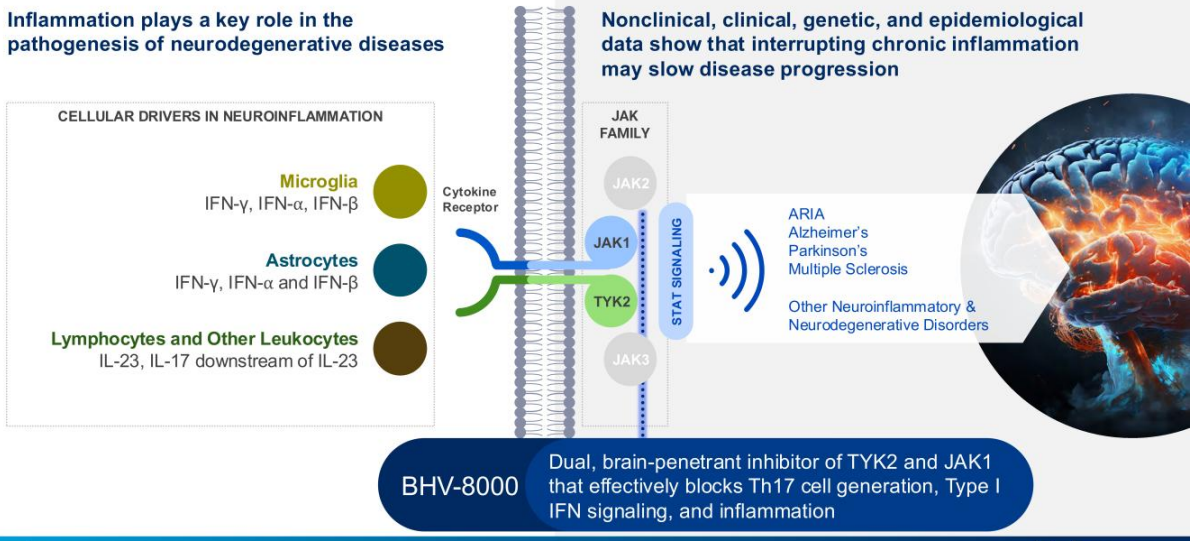
Innovative study design optimized for efficiency and sensitivity in detecting clinically meaningful change

BREAKING NEWS Phase 2/3 Parkinson's study initiated May 2025

BHV-8000: Brain-Penetrant TYK2/JAK1 Inhibitor With Potential to Treat Neuroinflammatory & Neurodegenerative Disorders

Inflammation plays a key role in the pathogenesis of neurodegenerative diseases

Nonclinical, clinical, genetic, and epidemiological data show that interrupting chronic inflammation may slow disease progression



BHV-8000 Dual, brain-penetrant inhibitor of TYK2 and JAK1 that effectively blocks Th17 cell generation, Type I IFN signaling, and inflammation

BHV-8000: Selectivity Predicts Improved Safety With Targeted Efficacy

Approved JAK Inhibitors — Significant Safety Risks Associated with JAK2 and/or JAK3 Inhibition						
		IC_{50} in nM				
Inhibitor	Status	JAK1 (autoimmune)	JAK2 (hematology)	JAK3 (leucocyte)	TYK2 (inflammation)	Safety
Tofacitinib[1]	Approved	15	77	55	489	Boxed Warning (MACE, malignancy, thrombosis, serious infections)
Baricitinib[1]	Approved	4	7	787	61	Boxed Warning
Upadacitinib[1]	Approved	47	120	2304	4690	Boxed Warning
Abrocitinib[1] (selective JAK1)	Approved	29	803	>15,000	1250	Boxed Warning* (*Development program suggests no increased clinical risk for these events)
Deucravacitinib[1] (selective TYK2)	Approved	>10,000	>10,000	>10,000	0.2	NO Boxed Warning

BHV-8000 Expected to Have a Favorable Safety Profile (avoids JAK2 and JAK3 inhibition)						
		IC_{50} in nM				
Inhibitor	Status	JAK1 (autoimmune)	JAK2 (hematology)	JAK3 (leucocyte)	TYK2 (inflammation)	Safety
BHV-8000	Phase 2 ready	4	118	>500	4	No expected risk of JAK2 and JAK3-related safety issues

IC_{50}, half maximal inhibitory concentration; **JAK**, Janus kinase; **MACE**, major adverse cardiac event; **TYK**, tyrosine kinase.

1. Wrobleski et al. *J Med Chem.* 2019;62(20):8973-8995.

BHV-8000: Demonstrates a Promising Phase 1 Profile

Phase 1 program completed

- Includes SAD/MAD study in healthy adults

Well-tolerated

- No SAEs or severe AEs
- No adverse laboratory trends related to study drug

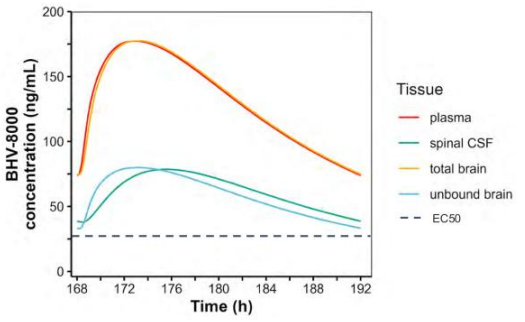
Evidence of target engagement

- Reduced plasma inflammatory cytokines downstream of TYK2 (IP-10, IFNβ) and JAK1 (hsCRP, IFNβ)

Robust brain penetration

- Exposures in CNS approximately 50% of plasma concentrations

BHV-8000 Brain Exposures at 20 mg QD

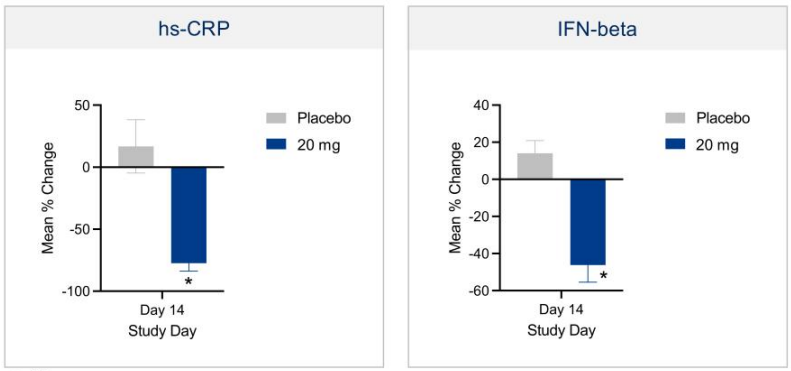


Modelling data

KEY POINT

Brain exposure sustained above target EC50s for 24 hours at clinically relevant doses

BHV-8000: Shows Evidence of Pharmacodynamic Effects



* p<0.05

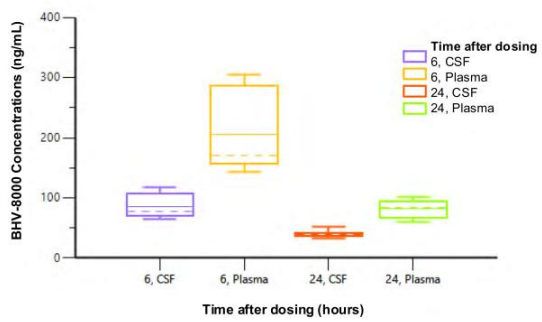
Preliminary Phase 1 data. **hs-CRP**, high-sensitivity C-reactive protein; **IFN-beta**, Interferon beta.

KEY POINT

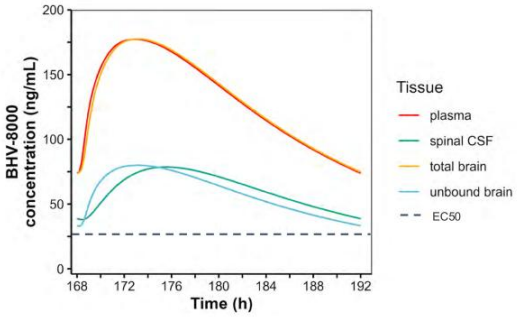
Pharmacodynamic data is indicative of target engagement in healthy subjects

BHV-8000: Demonstrates CNS Exposure

CSF and Plasma Concentrations (20 mg QD)



Brain PK sustained above EC50 for 24 hours at 20 mg QD



Modelling data

CSF, Cerebro Spinal Fluid

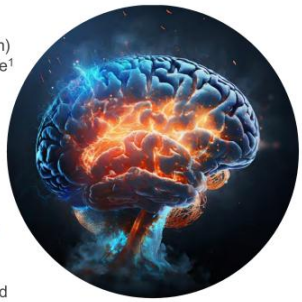
KEY POINT

Expected to have sustained brain exposures above EC50 (target engagement)

Parkinson's Disease Is an Autoimmune Disorder

PD meets criteria for autoimmunity based on pathophysiology and genetics

- Misidentification of self-proteins (α-synuclein) as foreign antigen triggers immune response[1]
- GWAS studies link PD risk to HLA gene variants involved in antigen presentation[2]



Epidemiology reveals increased risk of PD in individuals with other autoimmune diseases[3]

- Epi studies suggest immune dysfunction and inflammation are key to the development of PD[4]
- Reduction in rates of PD have been seen when this population is exposed to immune-modulating therapies[5]

PD animal models demonstrate immune dysregulation drives neurodegeneration[6]

- In mouse models, T cells specific to α-synuclein peptides can induce dopaminergic neuronal loss[7]
- Manipulation of immune components (T cells) affect α-synuclein-induced neurodegeneration[8]

PD patient samples and imaging exhibit characteristic proinflammatory signatures[9,10]

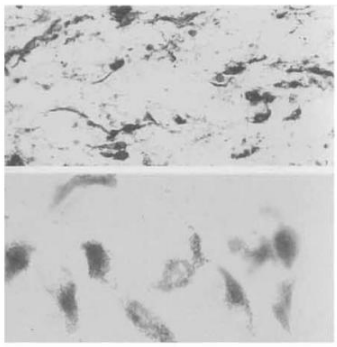
- Proinflammatory cytokines (e.g., IL-6, TNF-α, IFNɣ) are found in CSF and blood of PD patients[11]
- PD brains express high levels of HLA-DR+ reactive microglia[12]

1. Sulzer, *Nature* 2017; 2. Wissemann, *Am J Hum Genet* 2013; 3. Li, *Front Immunol* 2023; 4. Tansey, *Nat Rev Immunol* 2022; 5. Potashman, *Parkinsonism Relat Disord* 2025; 6. Roodveldt, *Brain* 2024; 7. Karikari, Brain Behav Immun 2022; 8. Williams, *Brain* 2021; 9. Yacoubian, *Mov Disord* 2023; 10. Pajares, *Cells* 2020; 11. Qu, *Nature* 2023; 12. McGeer, *Neurology* 1988.

BHV-8000: Clinical Data Supports Targeting Neuroinflammation in Parkinson's Disease

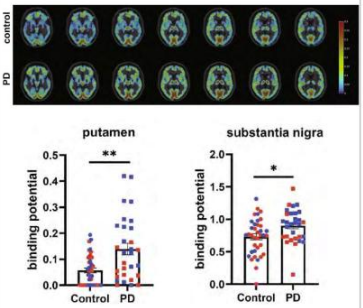
Post-Mortem Data[1]

PD patient brains express high levels of HLA-DR+ reactive microglia



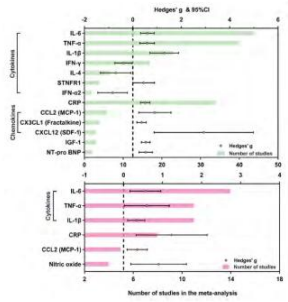
In Vivo Imaging[2]

^{18}F-DPA-714 TSPO imaging increased in early PD relative to healthy controls



In Vivo Cytokine Levels[3]

Elevated levels of pro-inflammatory cytokines (e.g., IL-1β, IL-6, TNF-α, IFN-ɣ) found in the CSF and blood of PD patients



1. McGeer PL, et al. *Neurology.* 1988 Aug;38(8):1285-91. 2. Yacoubian TA, et al. *Mov Disord.* 2023 May;38(5):743-754. 3. Qu Y, et al. *NPJ Parkinson's Dis.* 2023 Feb 4;9(1):18.

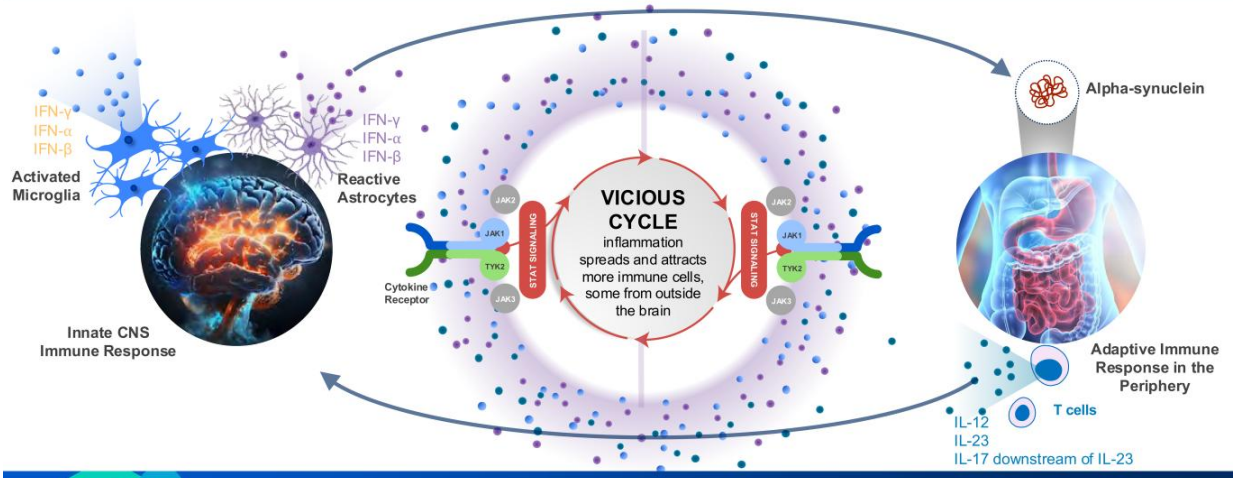
Real-World Analytics of Large Healthcare Database Show Parkinson's Disease Risk Reduction With TNF/IL-17 Targeting Therapies

- Biohaven conducted analysis using Komodo Health database (over 320 million patients since 2012) examining treatment with anti-TNF or anti-IL17 and incidence of PD
- Millions of patients over 8+ years of dosing captured key epidemiologic confirmation of the neuroinflammatory hypothesis
- Results support rationale for the effectiveness of BHV-8000 in treating PD

Treatment	PD Events	Person-years	Rate (per 100 person-years)	Adjusted IRR (95% CI)	P-value
Anti-TNF or Anti-IL-17 exposure	2,957	393,114	**0.66**	0.77 (0.74 – 0.80)	<0.0001
No Treatment	50,562	5,328,307	0.95		
Anti-TNF exposure	2,471	371,867	**0.66**	0.64 (0.52 – 0.80)	<0.0001
No Treatment	50,562	5,328,307	0.95		
Anti-IL-17 exposure	81	15,598	**0.52**	0.77 (0.78 – 0.81)	<0.0001
No Treatment	50,562	5,328,307	0.95		

IRR, incidence rate ratio; **TNF**, tumor necrosis factor.

TYK2/JAK1 Signaling Fuels Inflammation Driving Disease Progression in Parkinson's Disease



KEY POINT

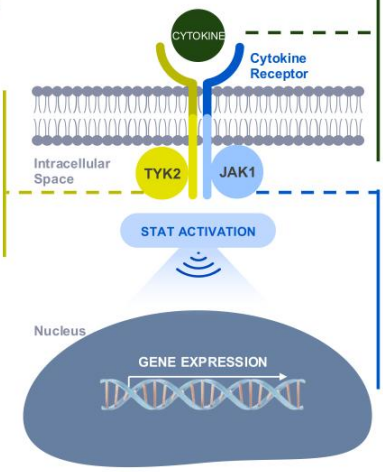
BHV-8000 treats both central and peripheral immune dysregulation underpinning Parkinson's

BHV-8000 Targets Both TYK2 and JAK1 to Control Immune Dysregulation in Parkinson's Disease

JAK-STAT SIGNALING PATHWAY

TYK2 PATHWAY
(IL-12, IL-23, etc.)

- Activates cytotoxic CD8 T cells and Th17 CD4 cells (which produce IL-17A) in the periphery
- IL-17A causes glial cell activation, release of neurotoxins such as TNFα, and disruption of the blood brain barrier[3]



SHARED TYK2 AND JAK1 PATHWAY
(IL-6, IFNα, IFNβ, etc.)

- Type-I IFN signaling is increased in post-mortem human PD brains[1]
- Type-I IFN modulates inflammatory response to α-syn in the gut, potentiating pathology along the gut-brain axis[2]

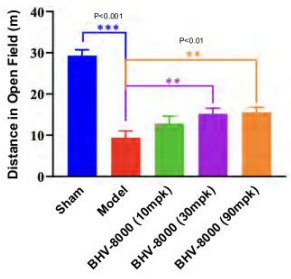
JAK1 PATHWAY
(IL-4, IFNγ, etc.)

- Activates CNS innate immune cells (microglia and astrocytes)
- Promotes T cell infiltration and upregulation of MHC II expression on glial cells and macrophages – exacerbating immune activation in the CNS[4]

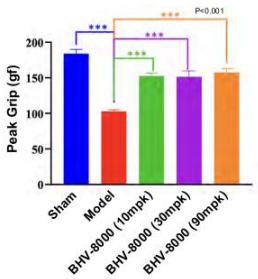
1. Main, Glia. 2016; 2. Waters, bioRxiv. Accessed May 15, 2025. https://www.biorxiv.org/content/10.1101/2024.05.05.592614v1. 3. Chen, *Front Aging Neurosci*. 2020; 4. Hong, *J Neuroinflammation*. 2024

BHV-8000: Efficacious in an AAV-α-synuclein Mouse Model of Parkinson's

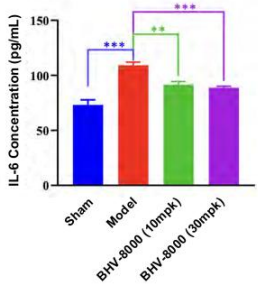
BHV-8000 Shows Dose Proportional Increase in Total And Corner Movement in an Open Field



BHV-8000 Shows an Increase in Grip Strength



BHV-8000 Treatment Significantly Decreased IL-6 Level in Str and SN Tissues of Mice



Note: IL-6 levels are elevated in PD patients

Phase 2/3 Study in Early Parkinson's Disease Initiated

POPULATION	Male and female adults living with early untreated PD
STUDY SIZE	550 participants randomized 1:1:1 (stratified by study site). 185 study sites across 13 countries (NA/EU)
TREATMENT	BHV-8000 10 mg or 20 mg or matching PBO PO once daily
TREATMENT DURATION	48-week double-blind treatment period. Completers to rollover into a 48-week extension study
KEY ENDPOINTS	**PRIMARY**: Time to qualifying worsening event on MDS-UPDRS Part II; **SECONDARY**: Change in MDS-UPDRS Part III, CGI-S, DaT-SPECT, PARCOMS composite scale, and Safety/tolerability

MDS-UPDRS, Movement Disorder Society – Unified Parkinson's Disease Rating Scale; **CGI-S**, Clinician Global Impression of Severity scale; **PARCOMS**, Parkinson's composite scale that includes most sensitive items from MDS-UPDRS and PDQ-39.

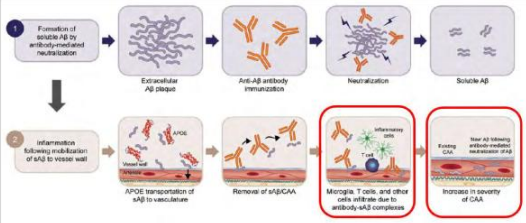
KEY POINT

Time-to-event primary endpoint enables fast and focused trial

BHV-8000: ARIA—A Potential Therapeutic Target for TYK2/JAK1 Inhibition

- ARIA events typically occur early after initiation of anti-amyloid mAb therapy.[1] The occurrence of ARIA can complicate the benefit-risk assessment in certain patient groups
- Reduction or elimination of ARIA should improve the uptake of anti-amyloid mAb therapies
- **Therapeutic hypothesis:**
  - TYK2/JAK1 inhibition reverses activated glial- and T-cell mediated pathology and general inflammation
  - Corticosteroids and other immunosuppressive drugs show benefit in treatment and reduce the risk for ARIA.[1,2,3] Generally, TYK2/JAK1 inhibition has a favorable benefit-risk profile over corticosteroids and other immunosuppressive drugs

Proposed Pathogenesis of ARIA[2]



- Antibody-dependent complement-mediated vascular injury, secondary to amyloid deposition in blood vessels[4]
- Loss of vascular integrity, impaired clearance and CAAri lead to vasculitis[2]
- Leakage of proteinaceous fluid and blood, results in ARIA-E or ARIA-H, respectively[2]
- Activated glial- and T-cell infiltration[2]
- Overexpression of C3 induces signaling through the JAK/STAT3 pathway[5]

[1]Cummings et al, *J Prev Alz Dis*. 2023;3(10):362-77; [2]Hampel e al, *Brain*. 2023 146;4414-24; [3]Regenhardt et al, *JAMA Neurol*. 2020 Oct;77(10)1-10; [4]Alzforum, 2023 https://www.alzforum.org/news/conference-coverage/aria-inflammatory-reaction-vascular-amyloid; [5]Yuan et al, *J Exp Clin Cancer Res*. 2020 V39:PMC6956509

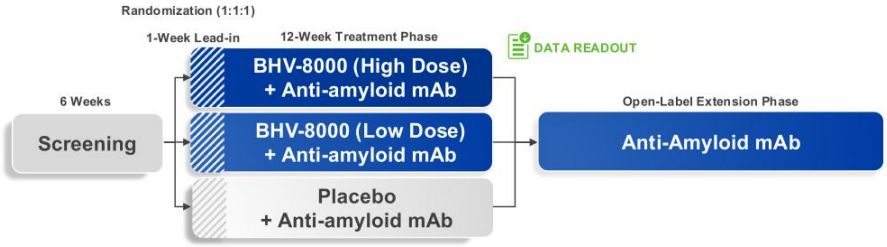
BHV-8000: Incidence of ARIA-E With Anti-Amyloid Therapy

Anti-Amyloid mAb, (n)	Overall, % (n)	APOE4/4, % (n)	APOE4/-, % (n)	Non-carriers, % (n)
EMERGE & ENGAGE TRIALS[1]				
Aducanumab[2] (1,029)	35.2 (362)	43.0[3] (290/674)		20.3 (72/355)
Placebo (1,076)	2.7 (29)	2.2[3] (16/742)		3.9 (13/334)
CLARITY-AD[4]				
Lecanemab (898)	12.6 (113)	32.6 (46/141)	10.9 (52/479)	5.4 (15/278)
Placebo (897)	1.7 (15)	3.8 (5/133)	1.9 (9/478)	0.3 (1/286)
TRAILBLAZER-ALZ2[5]				
Donanemab (853)	24.0 (205)	40.6 (58/143)	22.8 (103/452)	15.7 (40/255)
Placebo (874)	18 (2.1)	3.4 (5/146)	1.9 (9/474)	0.8 (2/250

- APOE4 carriers at increased risk for ARIA and accelerated progression of AD[6,7]
- Risk of ARIA can complicate the benefit-risk assessment of anti-amyloid mAbs, the only approved disease-modifying treatment for AD[8]

1. Salloway S., et al., JAMA Neurol. 2022;79(1):13-21. doi:10.1001/jamaneurol.2021.4161. 2. Results represent ARIA rates with aducanumab 10 mg/kg. 3. Represents ARIA-E rates with aducanumab (10 mg/kg) in APOE4 carriers (both hetero- and homo-zygotes); 4. van Dyck CH., et al., Lecanemab in early Alzheimer's disease. N Engl J Med. 2023;388(1):9-21. doi:10.1056/NEJMoa2212948. 5. Sims JR., et al., Donanemab in early symptomatic Alzheimer disease: the TRAILBLAZER-ALZ 2 randomized clinical trial. JAMA. 2023;330(6):512-27. doi:10.1001/jama.2023.13239. 6. Doran SJ., et al., Risk factors in developing amyloid related imaging abnormalities (ARIA) and clinical implications. Front Neurosci. 2024;18:1326784. doi: 10.3389/fnins.2024.1326784. 7. Hunsberger HC., The role of APOE4 in Alzheimer's disease: strategies for future therapeutic interventions. Neuronal Signal. 2019;3(2):NS201180203. doi: 10.1042/NS20180203. 8. Doran SJ., et al., Risk factors in developing amyloid related imaging abnormalities (ARIA) and clinical implications. Front Neurosci. 2024;18:1326784. doi: 10.3389/fnins.2024.1326784.

BHV-8000: Phase 2/3 Prevention of ARIA Study Design



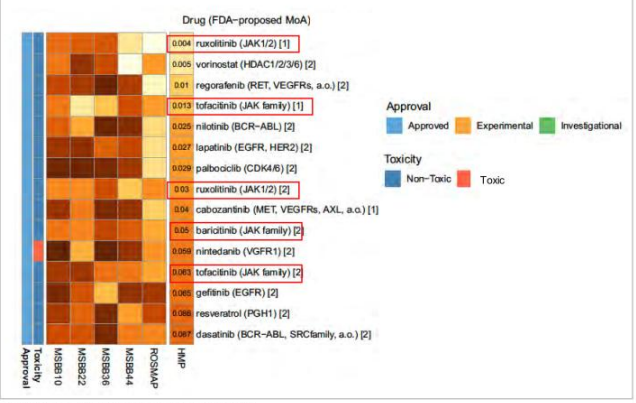
DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Male and female adults with early Alzheimer's disease who are APOE4 gene carriers
SAMPLE SIZE	450 participants (randomized 1:1:1 across 2 active and 1 placebo arm)
TREATMENT	BHV-8000 (high/low dose) vs. Placebo + anti-amyloid mAb
TREATMENT DURATION	1-week lead-in with BHV-8000 or Placebo; 12-week treatment period with BHV-8000 + anti-amyloid mAb; OLE with anti-amyloid mAb only
ENDPOINTS	Incidence of ARIA-E at Week 13; PK/PD; change in inflammatory and AD biomarkers, including brain amyloid

KEY POINT

Positive FDA feedback on novel prevention of ARIA indication, and on study design and clinical development plan

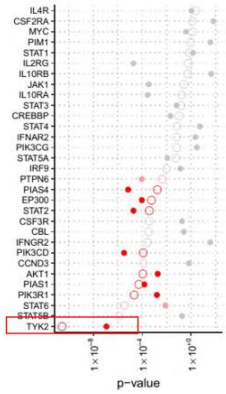
BHV-8000: Patient-Derived Evidence of TYK2/JAK1 Signaling in AD

Transcriptional Profiling Study of AD Patient Brains With Drug Mechanism Reveals Strongest Association For JAK Family Kinases



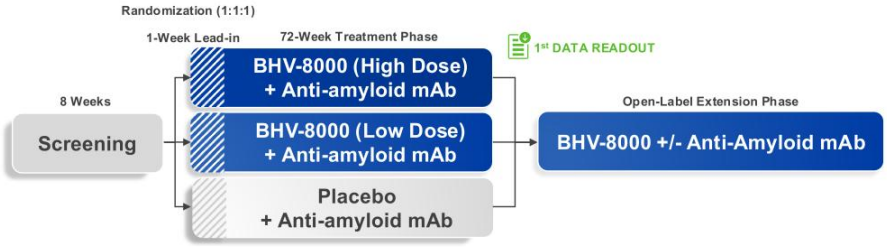
Rodriguez et al, *Nature Communications*. 2021 12:1033

TYK2 Is The Most Prominent Blood Leukocyte Gene In AD Patients



Nevado-Holgado et al, *Cells*. 2019 May 8;8(5):425

BHV-8000: Phase 2/3 Disease-Modifying Therapy Study in Early AD



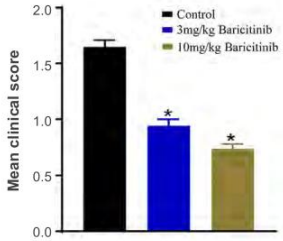
DESIGN	Randomized, double-blind, placebo-controlled trial
POPULATION	Male and female adults with early symptomatic Alzheimer's disease
SAMPLE SIZE	2,000 participants (randomized 1:1:1 across 2 active and 1 placebo arm)
TREATMENT	DBT Phase: BHV-8000 (high/low dose) vs. PBO + anti-amyloid mAb; OLE Phase: BHV-8000 +/- anti-amyloid mAb (IF amyloid PET "negative" can stop anti-amyloid mAb)
TREATMENT DURATION	1W lead-in with BHV-8000 or PBO; 72W DBT Phase; OLE Phase
ENDPOINTS	Primary: iADRS at W73; Secondary: CDR-SB, ADAS-Cog, MMSE, ADCS-iADL; Exploratory: Rates of ARIA, Change in inflammatory and AD biomarkers including brain amyloid / tau

fix

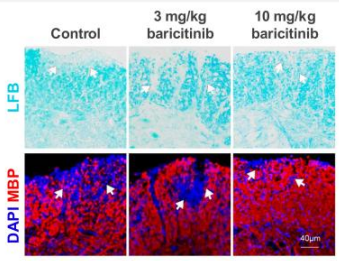
BHV-8000: TYK2/JAK1 Inhibition Is a Potential Treatment for Multiple Sclerosis

- **Genetic evidence:** Recent study found a protective genetic variation in the TYK2 gene that decreased signaling capacity in response to IL-12 and IL-23, reducing the function of TYK2, resulting in reduction in risk for developing MS[1]
- **Nonclinical data:** Suggests JAK/STAT pathway regulates differentiation and function of Th1 and Th17 cells which are essential for development of experimental autoimmune encephalomyelitis (EAE)[2]
- **Clinical data:** Supports the presence of abnormal immune activation in MS patients[3]

Baricitinib (JAK1/2 Inhibitor) Improved Mean Clinical Scores in Mice With EAE[2]

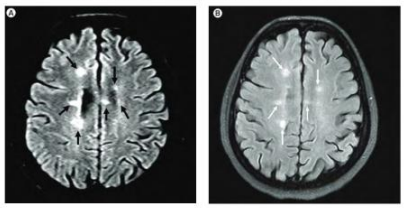


Baricitinib (JAK1/2 Inhibitor) Reduces Pathological Tissue Injuries In EAE Mice[2]

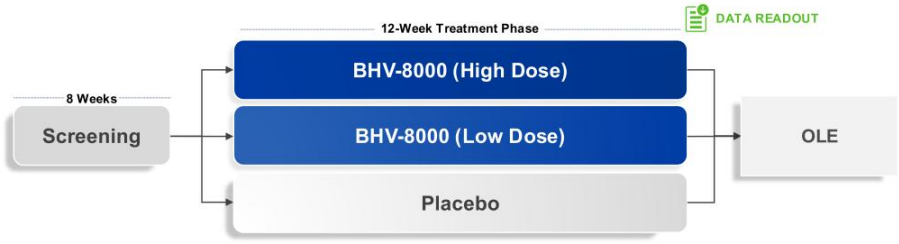


Secukinumab (IL-17A) Demonstrates an Effect in Relapsing Remitting MS[3]

Lesions regressed in MS patients 5 months of secukinumab treatment

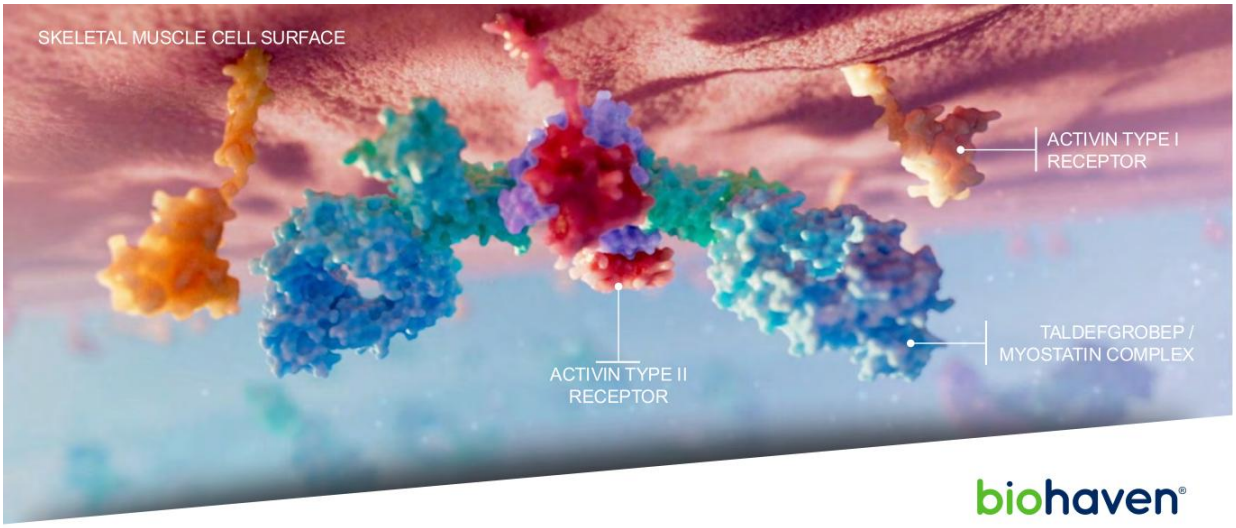


BHV-8000: Phase 2 Imaging POC Study in Relapsing Multiple Sclerosis



DESIGN	Randomized, double-blind, placebo-controlled Phase 2 imaging proof-of-concept study
POPULATION	Adults with relapsing multiple sclerosis (RMS)
SAMPLE SIZE	140 participants (randomized 2:2:1)
TREATMENT	BHV-8000 low dose or high dose versus placebo
TREATMENT DURATION	12-week double-blind phase followed by open label study
ENDPOINTS	Cumulative number of new gadolinium (Gd)-enhancing T1 lesions, total number of Gd-enhancing T1 lesions, number of new or enlarging T2 lesions, change in phase rim lesions, PK/PD

Preliminary clinical trial design



Myostatin Platform

Taldefgrobep Alfa for Obesity

Differentiated Pharmacology Key to Optimizing Benefit in Overweight and Obesity

- Taldefgrobep-myostatin complex competitively inhibits multiple key ligands from signaling through Activin II receptors (ActRII)
- Unique MOA leads to direct beneficial effects on both muscle and adipose tissues
- Safety profile established in diverse clinical populations (N >700) well-suited to overweight and obesity indication

Paradigm Shift to Improving Quality of Weight Loss

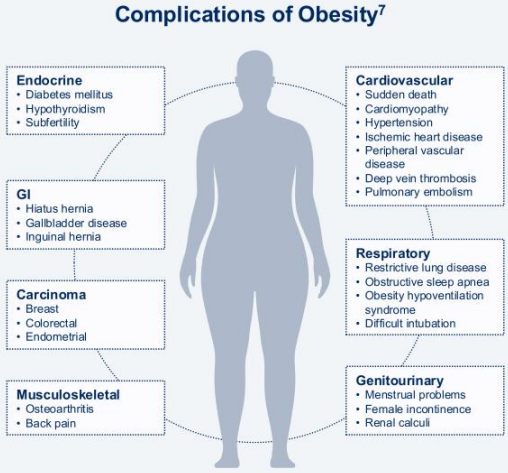
- Ability to lower total body weight by reducing fat mass while preserving lean muscle mass
- Use as monotherapy or in combination with Nutrient-Stimulated Hormone (NuSH)-based therapies, i.e., GLP-1 receptor agonists
- Weekly SC administration via off-the-shelf autoinjector, with potential for extended dosing intervals

KEY UPDATE

Phase 2 study in obesity planned for 2H 2025

Optimal Management of Obesity Remains a Critical Unmet Medical Need

- By 2030, 1 billion people worldwide will be living with obesity, including 50% of American adults[1]
- Obesity is a disease of excess and/or abnormal adipose tissue, not excess mass
- Incretin mimetics have revolutionized management of obesity, but present liabilities
  - Up to 40% of total body weight loss is lean mass[2]
  - Gastrointestinal side effects[3]
  - Reduced bone mass[4]
  - Two-thirds stop GLP-1 therapy within 1 year[5]
  - Two-thirds of lost body weight returns within 1 year of stopping GLP-1 therapy[5,6]



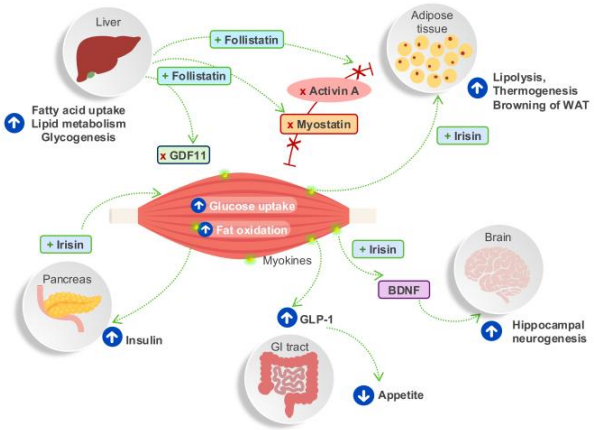
1 https://www.worldobesity.org/resources/resource-library/world-obesity-atlas-2022; Accessed 9-JAN-2025. 2. Wilding JPH et al, *N Engl J Med*. 2021;384(11):989-1002. 3. Wilding, et. al., Diabetes Obes Metab. 2022; 24(8):1553-64. doi: 10.1111/dom.14725 4. Hansen MS, et al., eClinicalMedicine. 2024;72:102624 5. Scientific American. What happens when you quit Ozempic or Wegovy? APR 2024. https://www.scientificamerican.com/article/you-quit-ozempic-or-wegovy-what-happens-next/ Accessed 9-JAN-2025. 6. Sikirica MV. Et al., Diabetes Metab Syndr Obes. 2017;10:403-12. 7. UpToDate. Overweight and obesity in adults: health consequences. https://www.uptodate.com/contents/overweight-and-obesity-in-adults-health-consequences. Accessed 9-JAN-2025.

Taldefgrobep Alfa: Novel MOA Optimizes Metabolic and Body Composition Change Important to those with Obesity

LOW MUSCLE MASS is associated with age-related cognitive decline[2] and increase in all-cause mortality[3]

HIGH MUSCLE MASS is associated with improvements in overall health and wellness

MYOKINES are important in the regulation of fat metabolism, inflammation, appetite, glucose control, bone density, and basal metabolic rate[1]



HIGH ADIPOSE MASS increases TGF-β ligands, leads to insulin resistance, and is a multifactorial driver of the morbidity of obesity

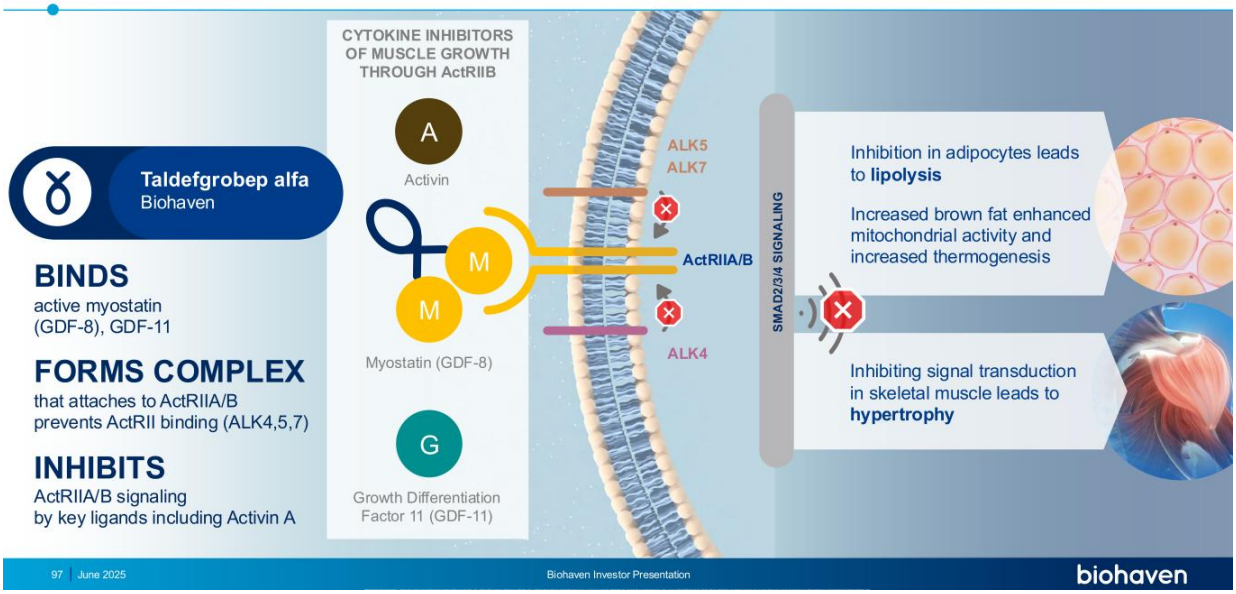
TALDEFGROBEP ALFA targets TGF-β ligands that signal through Activin II receptors including myostatin, GDF-11, and Activin A.[3-4] Inhibition of 3 ligands and ActRIIB optimizes muscle growth.[5]

1. Illustration adapted from Severinsen et al. Endocr Rev. 2020 Aug 1;41(4):594–609. 2. Daghlas et al. BMJ Med. 2023;2(1):e000354. 3 Lee et al. Exp Biol Med. 2018;243:1275-85. 4. Chen et al. Life Metabolism, 2024. 5. Latres, E., Mastaitis, J., Fury, W. *et al. Nat Comm* 8, 15153 (2017). **MSTN,** myostatin; **GDF11,** growth differentiation factor 11; **BDNF,** brain-derived neurotrophic factor.

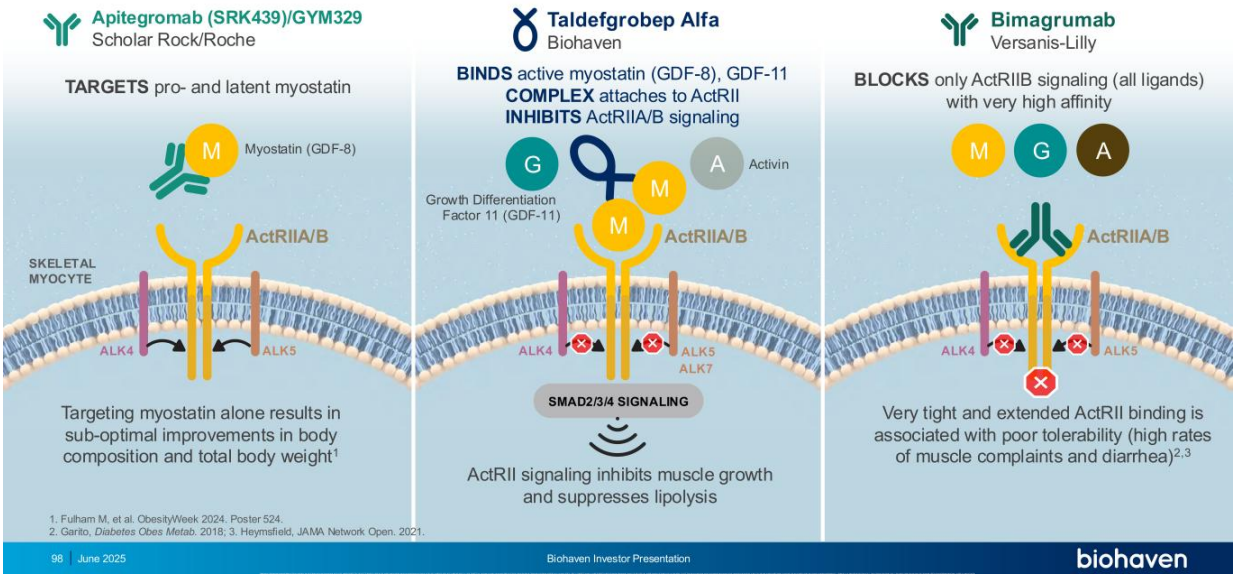
KEY POINT

Taldefgrobep alfa inhibits negative regulators of skeletal muscle and adipose tissue improving body composition and resulting in metabolic changes important to overall health and wellness

Taldefgrobep Has Direct Effects on Muscle and Fat — Optimizing Quality of Weight Loss



Taldefgrobep's Differentiated Approach Balances Efficacy and Safety



1. Fulham M, et al. ObesityWeek 2024. Poster 524.
2. Garito, *Diabetes Obes Metab.* 2018; 3. Heymsfield, JAMA Network Open. 2021.

Taldefgrobep: Highly Favorable and Differentiated Profile Within the "Myostatin Pharmacologic Class"

Apitegromab/GYM329
Scholar Rock/Roche

Pure Myostatin Agent

- Inhibits latent myostatin
- No direct ActRIIB receptor effects, so activity limited to PK of drug (limited PK/PD)
- Claims better safety due to selectivity
- Likely associated with decreased efficacy in muscle and adipose
- Requires IV infusion

Taldefgrobep alfa
Biohaven

Dual Myostatin Clearance and Activin Receptor Inhibition

- Binds active myostatin (pM), GDF-11 (pM) and Activin A (nM)
- Superior muscle growth to myostatin inhibition alone
- Accesses Activin A pharmacology
- Long lived T-alfa/myostatin complex reversibly binds ActRIIA/B inhibiting receptor signal transduction
- Low rates of AEs
- Favorable SC dosing

Bimagrumab
Versanis-Lilly

Activin Receptor Inhibitor

- Tight binding to and inhibition of ActRIIB receptors
- Superior muscle growth to myostatin inhibition alone
- Accesses Activin A pharmacology
- Long off-rate and tight binding results in **muscle spasms, fatigue, and diarrhea**
- Potent receptor inhibition results in lower FSH
- Requires IV infusion

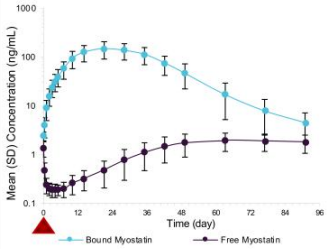
AE, adverse event; **FSH**, follicle stimulating hormone; **SC**, subcutaneous; **IV**, intravenous; **PK**, pharmacokinetics; **PD**, pharmacodynamics.

KEY POINT

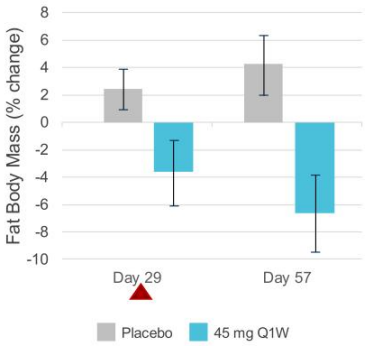
Taldefgrobep alfa potentially offers optimized efficacy, safety, and ease of use

Taldefgrobep Improves Body Composition in Non-Obese Adults with Potential for Monthly Dosing in Obesity

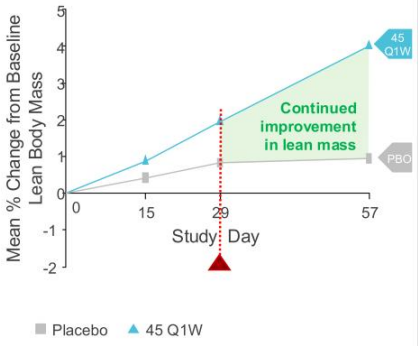
Peak Plasma Concentrations of **Taldefgrobep-Myostatin Complex** Occurs 20-30 Days Post Dose



Taldefgrobep Continued to Decrease **Total Fat Mass** Beyond the Dosing Period



Taldefgrobep Demonstrates Continued Improvement in **Lean Mass** in Healthy Adults at 30 Days Post Dosing



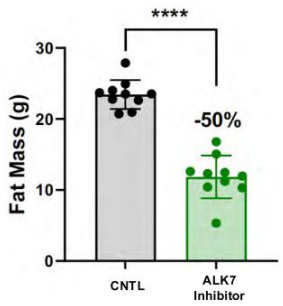
Last dose

Muntoni F. et al, *Neurol Ther.* 2024 Feb;13(1):183-219.

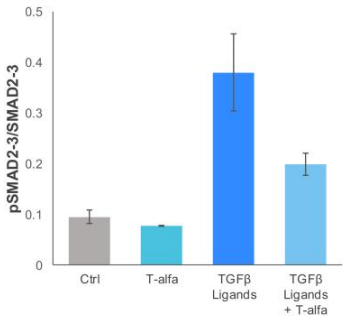
Taldefgrobep Stimulates Lipolysis and Decreases Fat Mass

- ALK7 activation blunts β-adrenoreceptor-mediated lipolysis and lipid oxidation in people with obesity[1]
- Reduction in ALK7 signaling can reduce fat mass in preclinical models[2]
- Taldefgrobep complex blocks ALK7-mediated SMAD2/3 signaling in adipocytes leading to increased lipolysis and a reduction in fat mass

ALK7 Inhibition Can Meaningfully Reduce Fat Mass in a DIO Mouse Model[1]



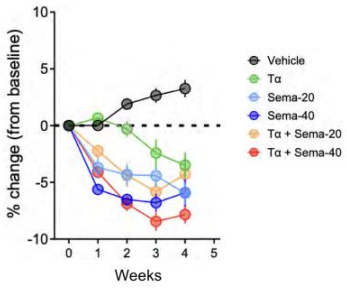
Taldefgrobep Blocks TGFβ-induced Signaling Through ALK7



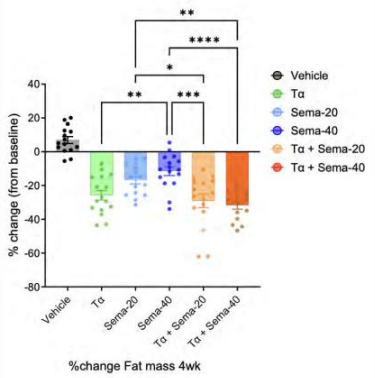
1. Guo. Elife 2014. 2. Ngai. Keystone Symposia on Obesity and Adipose Tissue, Poster. 2025

Taldefgrobep: Shows Greater Effect in Combination With Semaglutide than Semaglutide Alone in DIO Mice

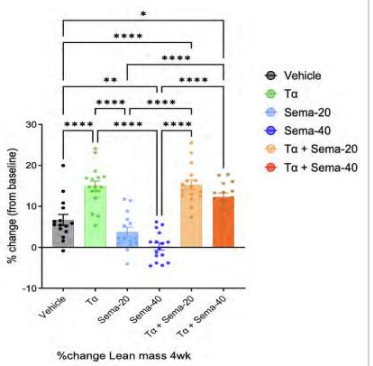
T-alfa Shows Weight Loss, and Combination with Semaglutide Shows **Higher Reduction in Body Weight** Than Semaglutide Alone



T-alfa and Combination Show **Greater Reduction in Fat Mass** Than Semaglutide Alone



T-alfa and Combination Show **Greater Increases in Lean Mass** Than Semaglutide Alone



Tα, taldefgrobep alfa; **DIO**, diet induced obesity.
* <= 0.05, ** <= 0.01, *** < 0.001 and **** <0.0001.

Taldefgrobep Monotherapy Offers Attractive Differentiation; but Also Potential for Combination With NuSH Therapies to Improve Quality of Weight Loss

FURTHER REDUCE

- ✓ Total body weight
- ✓ Total body fat
- ✓ Visceral adipose tissue
- ✓ Subcutaneous adipose tissue
- ✓ Hepatic fat
- ✓ Intramuscular fat
- ✓ HbA1c
- ✓ BP

Augment beneficial effects of NuSH therapies

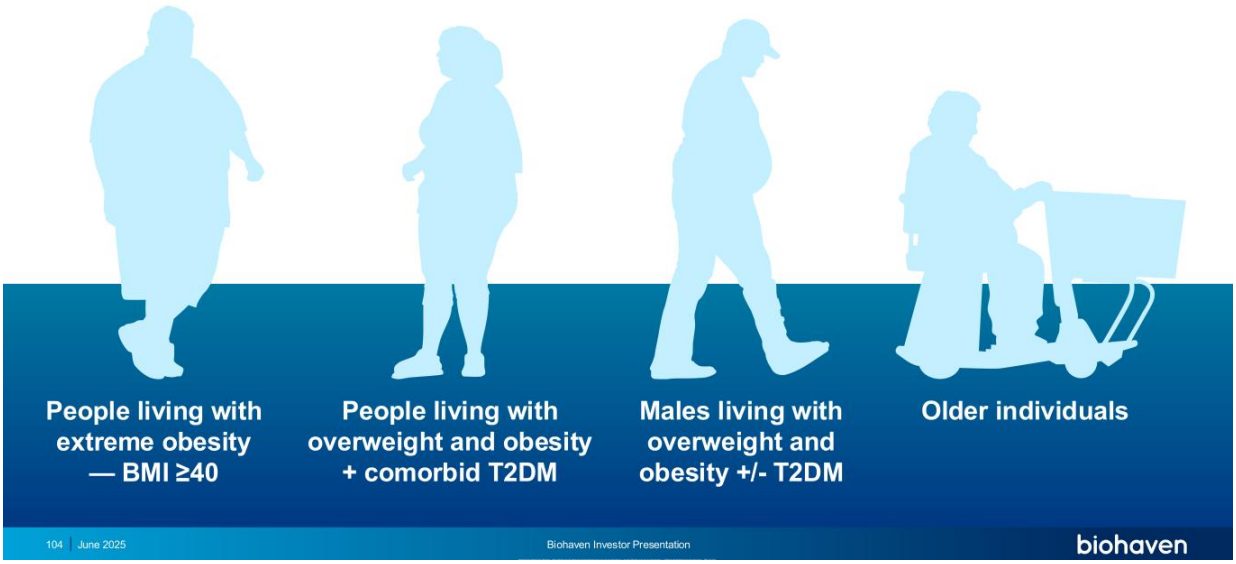
Address limitations of NuSH therapies

MITIGATE AGAINST

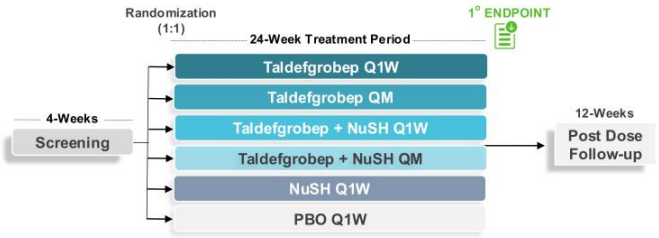
- ✓ Excess loss of lean mass
- ✓ Excess loss of bone mass
- ✓ Rapid weight rebound following interruption of dosing with NuSH therapies

NuSH Therapies, Nutrient-stimulating hormone therapies.

Populations That Can Benefit From Taldefgrobep + NuSH Combination



Taldefgrobep Phase 2 Combination Therapy Study in Overweight and Obesity



DESIGN	Randomized, double-blind, placebo-controlled dose-ranging trial
POPULATION	Male and female adults (18 – 75yo) living with overweight and obesity (BMI ≥27)
SAMPLE SIZE	Eligible participants randomized 1:1 (Sex [M/F] and BMI [<36, ≥36])
TREATMENT	Taldefgrobep SC once-weekly or once-monthly +/- NuSH-based therapeutic vs PBO
TREATMENT DURATION	24-week double-blind treatment period followed by 12-week post dose follow-up
ENDPOINTS	**Primary Endpoint**: Change in Total Body Weight (TBW) **Secondary Endpoints**: Lean Mass, Fat Mass, anthropometric measures, bone density, lipids, glycemic control, BP, PROs; PK/PD; safety/tolerability

Preliminary design

Design provides insight into key questions

- Impact of taldefgrobep monotherapy on body composition, TBW, metabolic endpoints in representative population
- Ability of taldefgrobep to provide additional benefits on top of NuSH therapies alone
- Ability of taldefgrobep to mitigate against reductions in lean mass associated with NuSH therapies

Taldefgrobep Alfa for Spinal Muscular Atrophy (SMA)

Differentiated Pharmacology Balancing Efficacy and Safety in SMA

- Taldefgrobep-myostatin complex competitively inhibits multiple key ligands from signaling through Activin II receptors (ActRII)
- Unique MOA leads to direct beneficial effects on both muscle and adipose tissues
- Safety profile established in diverse clinical populations (n >700)

Phase 3 Data in Spinal Muscular Atrophy

- Clinically meaningful improvements in motor function at all timepoints
- Robust target engagement (myostatin reduction)
- Beneficial impacts on body composition parameters (fat mass, lean muscle mass and bone density)
- Well-tolerated with 97% of participants continuing into optional OLE

KEY UPDATE

FDA interaction planned to discuss SMA registrational path in 1H 2025

High Unmet Need Still Remains For SMA

SOC treatments **target motor neurons** not muscle

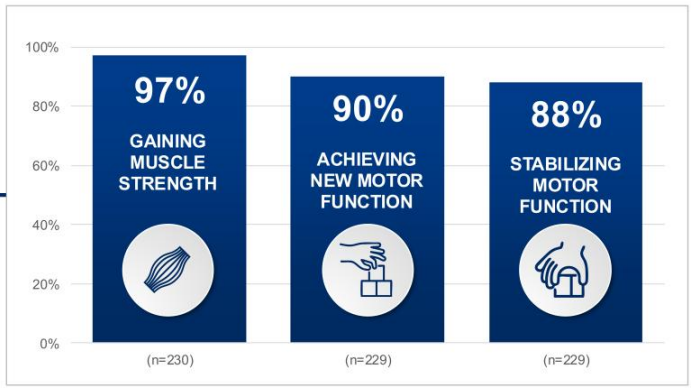
Patients on SOC treatment continue to experience **significant muscle weakness, reduced functioning and decreased quality of life**[1–7]

CALEB Living with SMA — Clinical trial participant on open label treatment

1. Mercuri E, et al. *Nat Rev Dis Primers*. 2022 Aug 4;8(1):52 . 2. Day JW, et al. *BMC Pediatr*. 2022;22(1):632. 3. Darras BT, et al. *N Engl J Med*. 2021;385(5):427-435. 4. Finkel RS, et al. *N Engl J Med*. 2017;377(18):1723-1732 5. Cure SMA. Accessed November 2024. https://www.curesma.org/wp-content/uploads/2018/01/SMA-VoP-for-publication-1-22-2018.pdf. 6. Darras BT, et al. *N Engl J Med*. 2021;385(5):427-435. 7. Finkel RS, et al. *N Engl J Med*. 2017;377(18):1723-1732.

97% of Adults With SMA Hope New Therapies Will Help Them Gain Muscle Strength

"What are your most significant current unmet needs that you hope new therapies would address?"[1]



1. Cure SMA. Accessed November 2024. https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf.

KEY POINT

Muscle-targeting therapy is needed on top of SOC treatment (SMN upregulation)

Taldefgrobep Alfa: Well-Characterized Clinical Profile

CLINICAL DEVELOPMENT

>700 trial participants treated (adults and children) across 6 studies

- Completed: 3 Phase 1 trials (SAD/MAD, SC BA, autoinjector BE); 1 Phase 1b/2 trial in DMD and 1 Phase 2/3 trial in DMD
- Ongoing: Phase 3 trial in SMA

PHARMACOLOGY

Demonstrated target engagement and increased muscle mass

- Explored broad range of doses (4 mg to 180 mg SC QW) for up to 120 weeks of repeat dosing
- Demonstrated target engagement with dose-related free myostatin suppression
- Noted accumulation of drug-myostatin complex with sustained pharmacological activity beyond dosing period in healthy adults
- Demonstrated increased muscle in healthy adults, DMD and SMA

SAFETY

Safe and well-tolerated to date with differentiated profile

- Low rates of SAEs, and few AEs leading to discontinuation
- Does not have AEs commonly reported in other drugs in the class (e.g., GI-related and muscle-related side effects)

Taldefgrobep Alfa Achieved Robust Target Engagement

Free Myostatin Mean ng/mL, (SD)			
	Baseline	**Week 12**	**Week 48**
Taldefgrobep alfa	0.233 (0.118)	undetectable	undetectable
Placebo	0.259 (0.147)	0.267 (0.133)	0.255 (0.112)

Myostatin measured by commercial ELISA kit with BLQ < 0.125 ng/mL

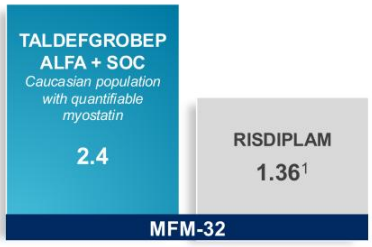
One third of taldefgrobep alfa participants had undetectable myostatin at baseline (pre-treatment)

KEY POINT

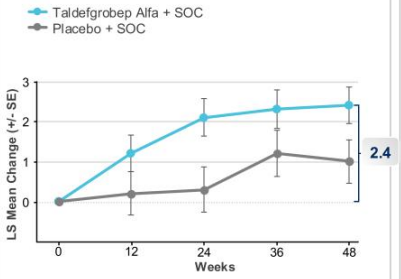
Taldefgrobep alfa reduced free myostatin levels below detection in myostatin-positive participants

Taldefgrobep Alfa Treatment Effect on MFM-32 Is Additive to SOC

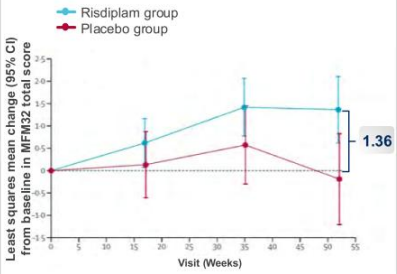
Change From Baseline on MFM-32



Taldefgrobep Alfa



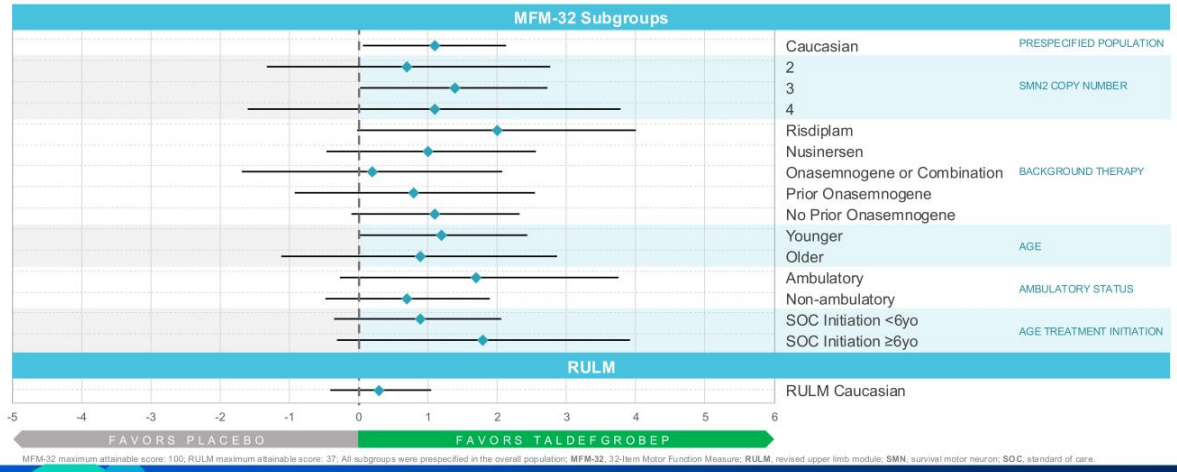
Risdiplam[1]



LS, least squares; **MFM-32**, 32-Item Motor Function Measure; **SE**, standard error; **SOC**, standard of care.
1. Mercuri E, et al. *Lancet Neurol.* 2022 Jan 21(1) 42-52 2

T-ALFA

Treatment Difference Favors Taldefgrobep Alfa in Caucasian Population: MFM-32 Subgroups and RULM at Week 48

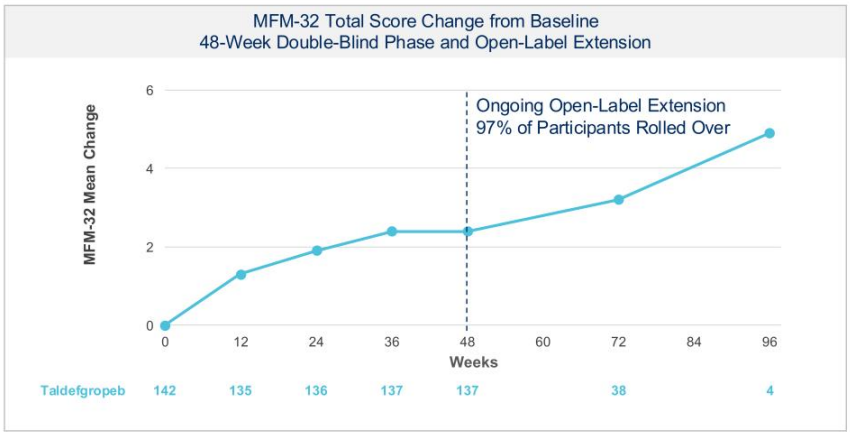


MFM-32 maximum attainable score: 100; RULM maximum attainable score: 37; All subgroups were prespecified in the overall population; **MFM-32**, 32-Item Motor Function Measure; **RULM**, revised upper limb module; **SMN**, survival motor neuron; **SOC**, standard of care.

KEY POINT

Results favored taldefgrobep alfa on the primary outcome in participant groups not enrolled in other studies

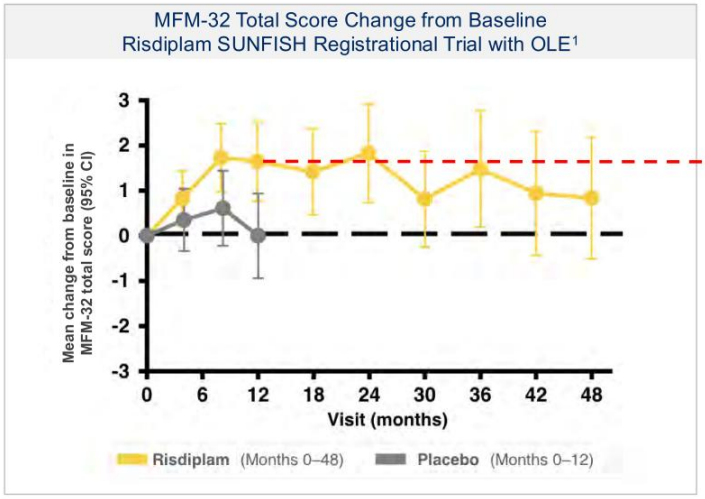
Taldefgrobep Alfa Clinically Meaningful Treatment Effect Continues to Increase After 1 Year of Treatment in Overall Population



KEY POINT

Supports treatment benefit in taldefgrobep alfa-treated participants vs expected MFM-32 trajectory

MFM-32 Trajectory Expected to Decline on Stable SOC Treatment



MFM-32, 32-Item Motor Function Measure; **CI**, confidence interval; **SOC**, standard of care; **OLE**, open-label extension.
1. L Servais, et al. Presented at: AAN; April 22-27, 2023; Boston, MA.

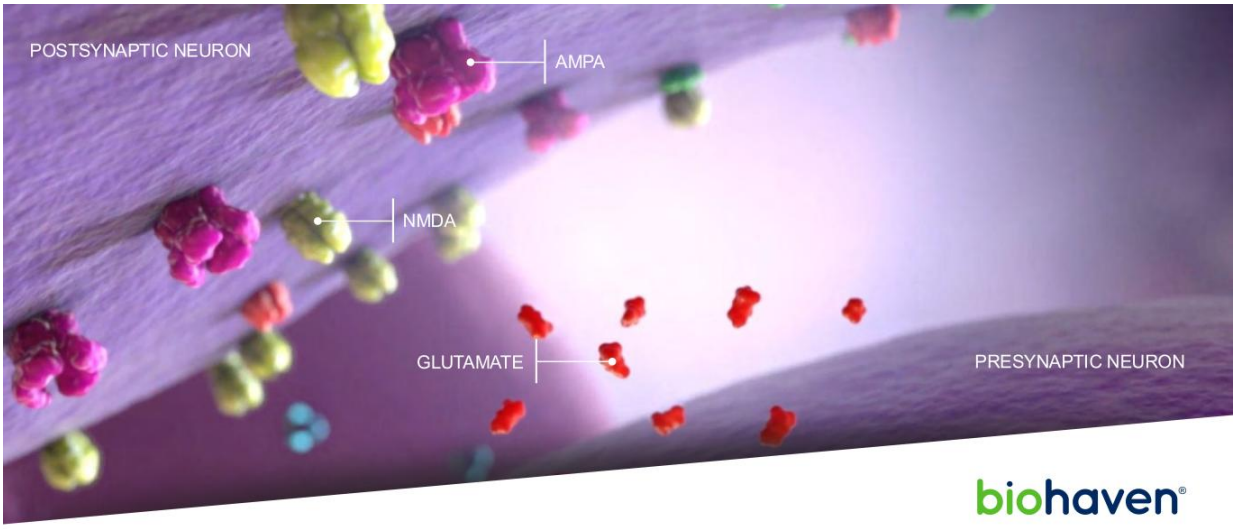
Taldefgrobep Alfa Safe and Well-Tolerated in Phase 3 SMA Study: TEAEs Reported in ≥ 10% of Participants Overall During DB Phase

	Placebo (n=73)	Taldefgrobep Alfa (n=143)	Overall (n=216)
	Participants with Event: n (%)	Participants with Event: n (%)	Participants with Event: n (%)
Upper respiratory tract infection	21 (28.8)	37 (25.9)	58 (26.9)
Pyrexia	21 (28.8)	27 (18.9)	48 (22.2)
Nasopharyngitis	19 (26.0)	23 (16.1)	42 (19.4)
Cough	9 (12.3)	23 (16.1)	32 (14.8)
Vomiting	9 (12.3)	22 (15.4)	31 (14.4)
Injection site erythema	4 (5.5)	26 (18.2)	30 (13.9)
Headache	9 (12.3)	20 (14.0)	29 (13.4)

DB, double-blind; **SAE**, serious adverse event; **TEAE**, treatment emergent adverse event.

KEY POINTS

- All SAEs judged not related to study drug
- Low rate of TEAEs leading to discontinuation: 4 participants on taldefgrobep (2.8%); 1 on placebo (1.4%)


POSTSYNAPTIC NEURON
AMPA
NMDA
GLUTAMATE
PRESYNAPTIC NEURON
biohaven®

Glutamate

Significant Unmet Need in SCA With No Available Treatment

- Rare, relentlessly progressive, inherited neurodegenerative disease
- ~15,000 patients in US

US Priority Review of NDA With FDA Is Underway

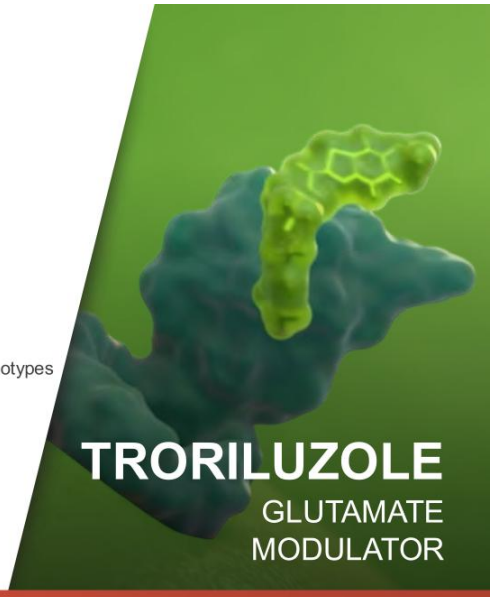
- Completed mid-cycle review and inspections of HQ and clinical trial sites
- Preparing for Advisory Committee and late-cycle meetings
- PDUFA date 2H 2025

Efficacy and Safety Demonstrated Over 8 Years

- Study 206-RWE met prespecified primary endpoint (f-SARA at Year 3) in all SCA genotypes
- Additional confirmatory evidence from Study 206-RWE, Study 206 and Study 201

Biohaven Will Be Ready to Serve Patients Upon Anticipated Approval

- EAP currently expanding across multiple sites in the US due to patient demand
- Preparing for US commercial launch in 2H 2025 in event of approval

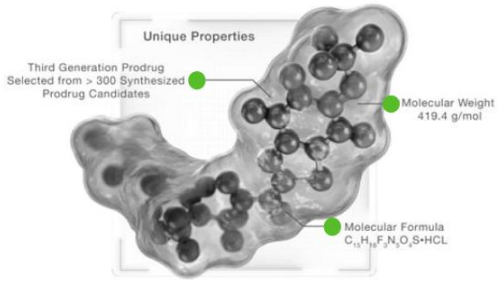


BREAKING NEWS

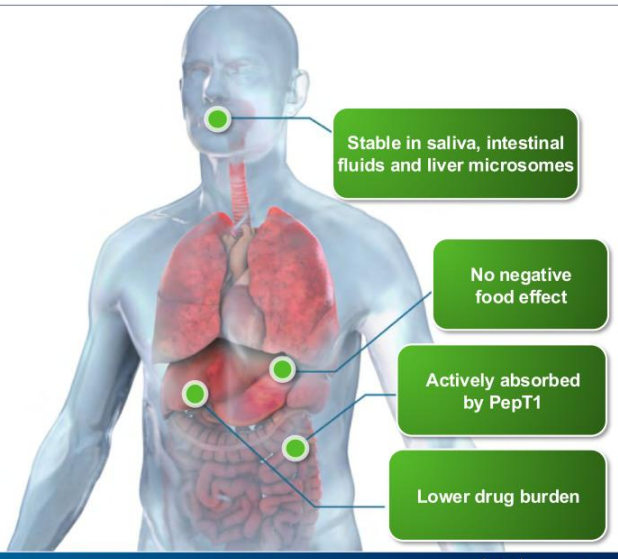
Troriluzole US NDA currently under priority review for SCA — 2H 2025

Troriluzole Was Rationally Designed to Optimize Therapy

TRORILUZOLE



- ✓ Improved absorption
- ✓ Reduced drug burden
- ✓ Favorable safety profile
- ✓ Enhanced bioavailability
- ✓ Reduced first pass metabolism
- ✓ Once-daily dosing



PepT1, peptide ttransporter 1.

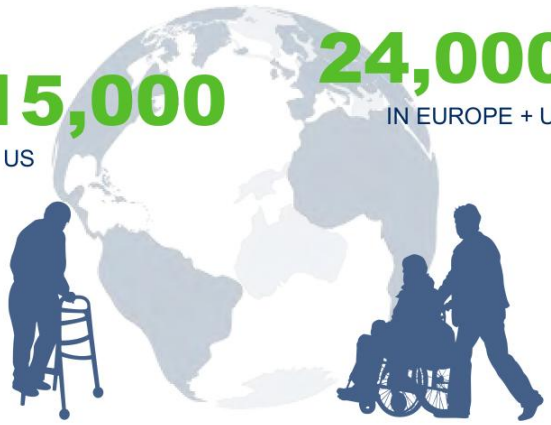
SCA: Rare Progressively Debilitating and Fatal Neurodegenerative Disorder with No Approved Treatment

- Autosomal dominant, progressive, neurodegenerative disease with multiple genotypes[1–3]
- Onset in early adulthood with symptoms leading to severe disability and premature death[3]
- High unmet need and no approved therapies[1,2]

SCA Prevalence[4]

15,000
IN US

24,000
IN EUROPE + UK



f-SARA: Neurologist-Assessed Scale that Tracks SCA Disease Progression

- Measures 4 core functional items that are clinically meaningful and reflect hallmark symptoms of SCA5
- Individual items rated 0–4 with total score range 0–16
- Generally increases (worsens) 0.5 points annually
- Developed with FDA input
- Psychometric and qualitative validation performed according to FDA guidance[5,6]

KEY POINT f-SARA is an approvable endpoint in SCA

Troriluzole Restores Glutamate Homeostasis and Delays Disease Progression Across SCA Genotypes

Strong Mechanistic Rationale

Preclinical and clinical evidence for role of glutamate in SCA

Clinically Meaningful Effect on f-SARA

Clear evidence for troriluzole benefit in SCA

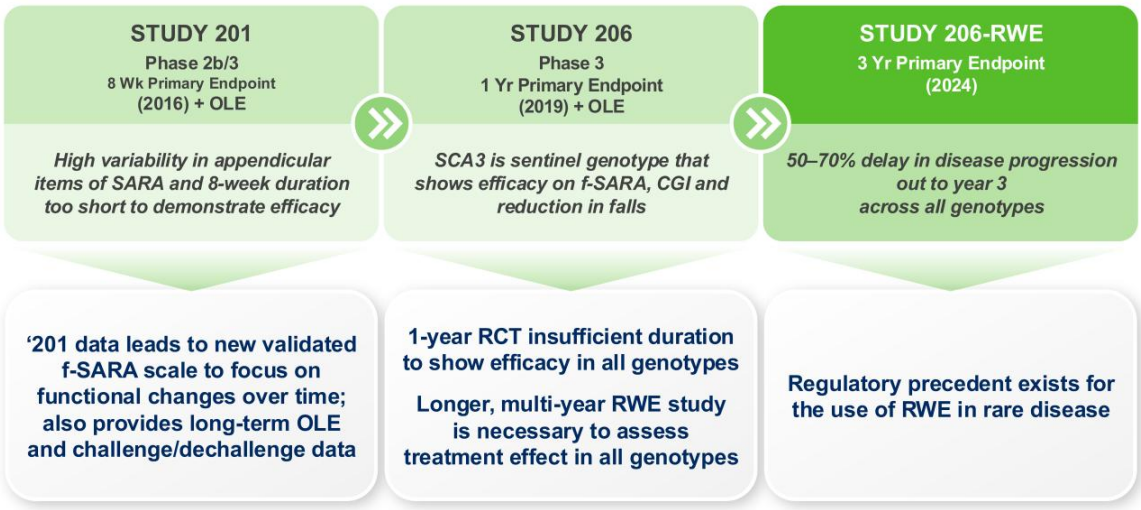
Impact on Disease Progression

Observed across the 8-year SCA development program

Reduction in Falls

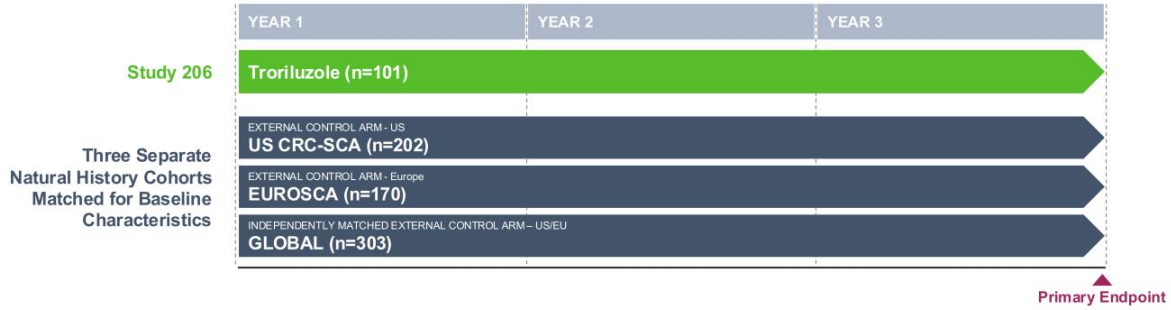
Falling is cardinal feature of SCAs leading to injury and reduced QoL

SCA is Slowly Progressive Disease with Multiple Genotypes: Requires Multi-Year Follow-up to Assess Efficacy Across Genotypes



RWE Study Was Designed With FDA Input & Following RWE Guidance

3-year real-world evidence protocol with external control using propensity score matching



PRIMARY ENDPOINT

f-SARA Change from baseline at 3 years in troriluzole-treated subjects vs untreated subjects from US Natural History control (CRC-SCA)

SECONDARY ENDPOINTS

f-SARA Change from Baseline:

- at 2-and 1-years vs US Natural History external control (CRC-SCA)
- at 3-, 2-, and 1-years vs EU Natural History external control (EUROSCA)
- at 3-, 2-, and 1-years vs Global, US and EU Natural History external control (CRC-SCA and EUROSCA)

RWE Study Designed With FDA Feedback to Reduce Bias

Biohaven Proposal	FDA Feedback	BHV4157-206-RWE Protocol
Analyze new data from subjects completing 3-years of treatment	**Follow Industry Guidance for RWE***	Regulatory precedent for NDA approval based on RWE and external control for analysis of 3-year endpoint. Inclusion of new 3-year completers nearly doubles the sample size and thus increases power and precision as these data were not previously available
Submit SAP for FDA review	**Submit both Protocol and SAP for FDA review prior to database lock**	Submitted Protocol and SAP to FDA with prespecified endpoints and analysis plan based on FDA input ahead of database lock; **Biohaven adopted all feedback proposed by the FDA**
Use composite scale as primary outcome measure	**Use f-SARA as primary outcome measure**	f-SARA used as primary outcome measure; a reliable and validated scale to measure clinically meaningful change in function in SCA; designed with FDA input that is objective and minimizes effort dependence
Use global SCA Natural History cohorts as external control	**Use US SCA Natural History cohort as external control for primary analysis**	Minimizes potential for bias by ensuring the Biohaven trial & US SCA Natural History study conducted by same sites/investigators, evaluating similar scales, over similar time period, with same population, on same standard of care treatment
Use MAIC analysis as primary	**Use Propensity Score Matching (PSM) for primary analysis**	Minimizes potential for confounding bias by balancing baseline characteristics between treatment group and external control; Used in other NDAs leveraging RWE**
Genetic risk factors not included in matching	**Match populations based on trinucleotide repeat length for primary analysis**	Minimizes potential for bias by further matching treatment group and external control based on additional genetic factors associated with disease burden

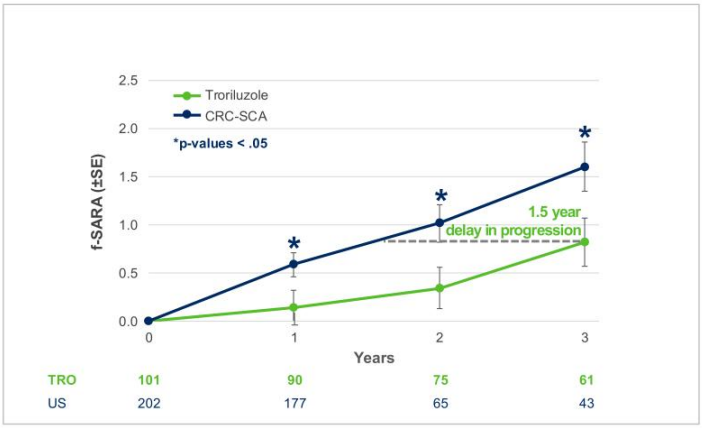
*Guidance for Industry Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision Making for Drug and Biological Products https://www.fda.gov/media/171667/download
**Lynch DR, et. al. Propensity matched comparison of omaveloxolone treatment to Friedreich ataxia natural history data. Ann Clin Transl Neurol. 2024 Jan;11(1):4-16

Demographic and Baseline Characteristics

	BHV4157-206	CRC-SCA	EUROSCA
n	105	446	358
Age (years), n	105	434	358
mean (SD)	47.6 (13.1)	51.6 (13.8)	47.3 (12.7)
median (range)	49.0 (18, 73)	52.0 (0, 89)	47 (18, 84)
Sex, n	105	446	358
Male (%)	47 (45)	200 (45)	171 (48)
Female (%)	58 (55)	246 (55)	187 (52)
Age at symptom onset (years)			
mean (SD)	37.7 (12.4)	41.2 (13.9)	36.7 (11.8)
median (range)	38 (10, 71)	41 (0, 76)	37 (7, 76)
Genotype (%)			
SCA1	15 (14)	66 (15)	102 (29)
SCA2	31 (30)	94 (21)	141 (39)
SCA3	41 (39)	153 (34)	115 (32)
SCA6	6 (6)	95 (21)	0
SCA7	5 (5)	5 (1)	0
SCA8	3 (3)	19 (4)	0
SCA10	3 (3)	6 (1)	0
Multiple	1 (1)	3 (1)	0
f-SARA			
mean (SD)	4.95 (1.6)	3.97 (3.5)	5.03 (4.1)
median (range)	4.00 (2,10)	3.00 (0,16)	4.00 (0,16)

Full Analysis Set

Troriluzole vs Matched US Natural History External Control Shows Slowing of Disease Progression out to Year 3

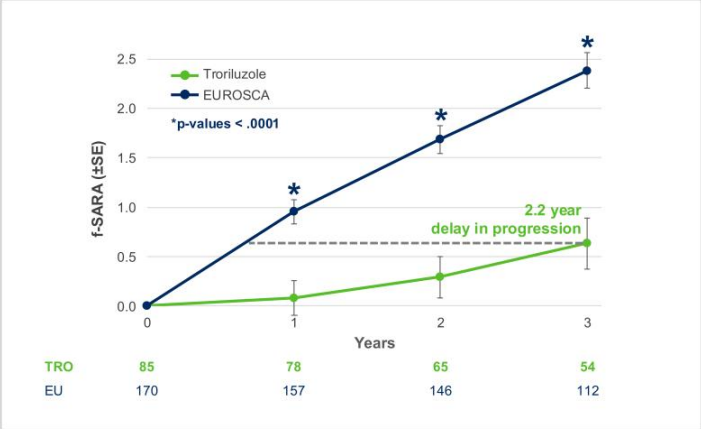


CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia; **LSM**, least squares mean; **PSM**, Propensity Score Matching

KEY POINT

Troriluzole reduced SCA disease progression by 50%

Troriluzole vs Independent Matched EU Natural History External Control Shows Slowing of Disease Progression out to Year 3

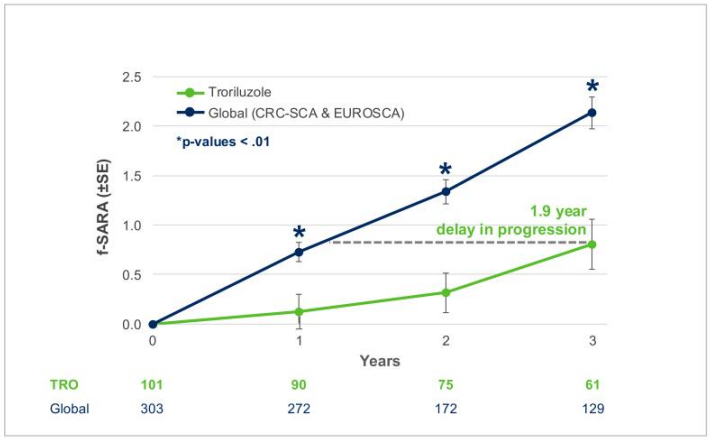


CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia; **LSM**, least squares mean; **PSM**, Propensity Score Matching

KEY POINT

Troriluzole reduced SCA disease progression by 70%

Troriluzole vs Matched Global Natural History External Control Shows Slowing of Disease Progression out to Year 3



CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia; **LSM**, least squares mean; **PSM**, Propensity Score Matching

KEY POINT

Troriluzole reduced SCA disease progression by 60%

Troriluzole Met 9 Prespecified Consecutive Hierarchical Endpoints Demonstrating Robust and Durable Treatment Benefit Over 3 Years

	f-SARA at Year	p-value
US External Control (CRC-SCA)	3	<0.05
	2	<0.05
	1	<0.05
Europe External Control (EUROSCA)	3	<0.0001
	2	<0.0001
	1	<0.0001
Global External Control (CRC+EURO)	3	<0.0001
	2	<0.0001
	1	<0.003

CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia; **Global**, CRC-SCA & EUROSCA.

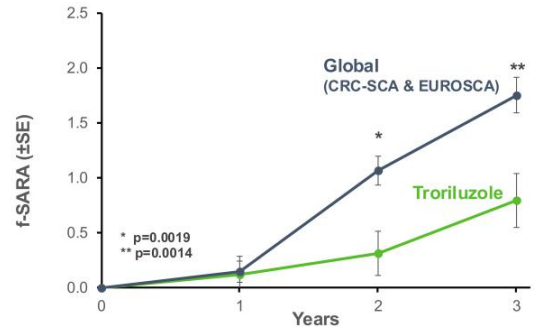
Untreated SCA Patients Have Higher Likelihood of Significant Worsening

	Odds Ratio of f-SARA ≥2-Point Worsening in Untreated	p-value
US External Control vs Troriluzole*	**2.4**	0.0359
EU External Control vs Troriluzole	**6.1**	<0.0001
Global External Control vs Troriluzole	**4.1**	<0.0001

*Prespecified

KEY POINT f-SARA ≥2-point change at 3 years represents a clearly defined worsening of SCA disease

Anchoring Analysis Confirms Need for 3 Year Study to Overcome Heterogeneity in SCA Progression Patterns



Troriluzole (n)	101	90	75	61
Global (n)	273	273	162	129

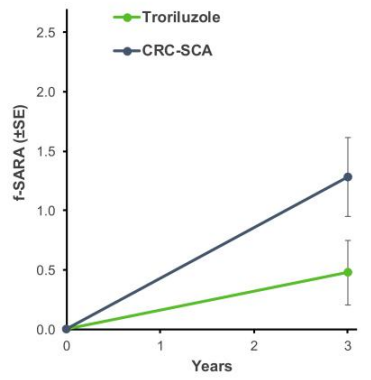
- External control was anchored to Study 206 placebo progression rate over 1-year using PSM
- Anchored analysis shows efficacy at Years 2 and 3 consistent with primary results from Study 206-RWE
- Addresses any potential bias introduced by differences in progression rates between RWE and Study 206 patients
- Supports reliability and interpretability of using external control arm in assessing troriluzole

CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia; **PSM**, Propensity Score Matching.

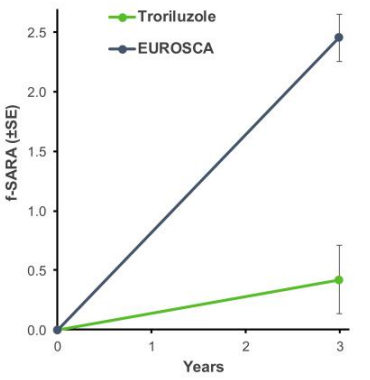
KEY POINT

Anchoring analysis shows RWE findings are independent and highly consistent with 1-year RCT (Study 206) and closely reflects potential results of a 3 Year RCT study

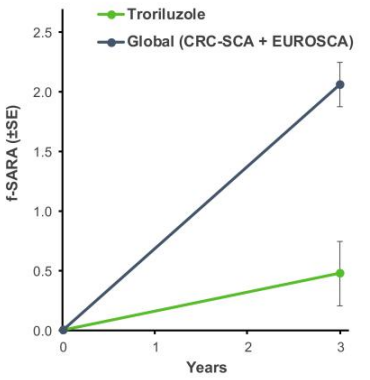
New Data From Subjects Completing 3 Years of Treatment Was Consistent With Primary Results in Each External Control Arm



Troriluzole (n)	21	21
CRC-SCA (n)	202	43



Troriluzole (n)	19	19
EUROSCA (n)	170	112



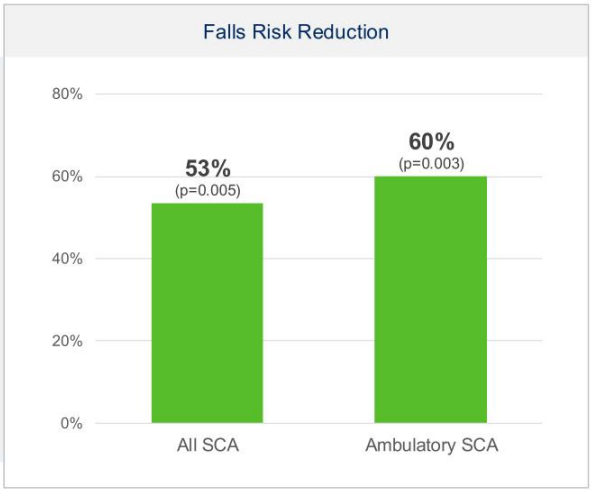
Troriluzole (n)	21	21
Global (n)	303	129

CRC-SCA, Clinical Research Consortium for SCA; **EUROSCA**, European registry of SCA; **f-SARA**, Functional Scale for the Assessment and Rating of Ataxia.

Troriluzole Substantially Reduced Fall Risk in Double-Blind Phase

Burden of Falls in SCA[9–10]

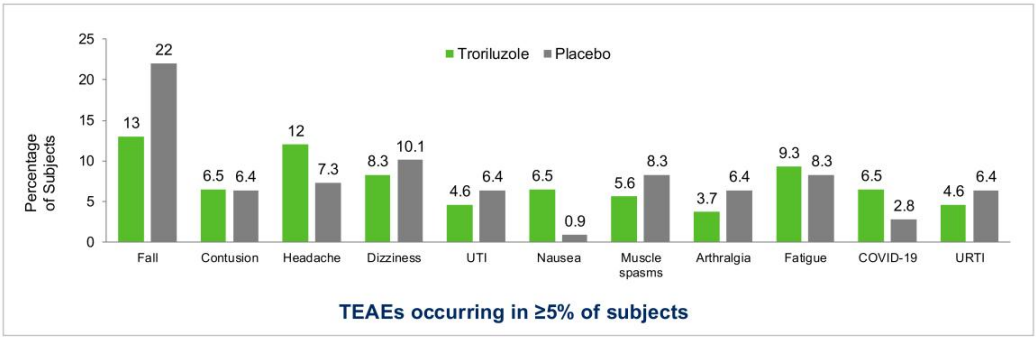
- Most SCA patients (74–84%) report falling in the preceding 12 months
- Falling is associated with a high rate of injury (74%)
- Frequent fallers report more fall-related injuries
- Fall frequency decreases when patients become wheelchair dependent or immobile



* Study BHV4157-206 double-blind phase results; Falls were captured in Study BHV4157-206 as adverse events if reported as "worsening falls" or if the fall resulted in an injury. For the analysis, a generalized linear model was fit using a Poisson family model with a log link function.
** Ambulatory SCA is defined as All SCA subjects who could ambulate without constant assistance (scoring 1 or 2 on the gait item of the f-SARA) at baseline

Troriluzole Was Well-Tolerated in Clinical Trials

	Troriluzole N=108	Placebo N=109
Serious TEAE	**6 (5.6)**	8 (7.3)
Severe TEAE	**3 (2.8)**	8 (7.3)
TEAE Leading to Discontinuation	**5 (4.6)**	5 (4.6)



TEAEs occurring in ≥5% of subjects

Study BHV4157-206 double-blind phase results; falls were captured as adverse events if reported as "worsening falls" or if the fall resulted in an injury.

Troriluzole Demonstrates Compelling Treatment Effect and Meaningful Delay in SCA Disease Progression

Clear Impact on Disease Progression

- 50–70% slowing of disease progression, representing 1.5–2.2 years delay in disease progression over 3-year RWE Study
- Multiple sensitivity analyses confirm reliability and interpretability of study results

Consistent Treatment Effect Over Three Studies

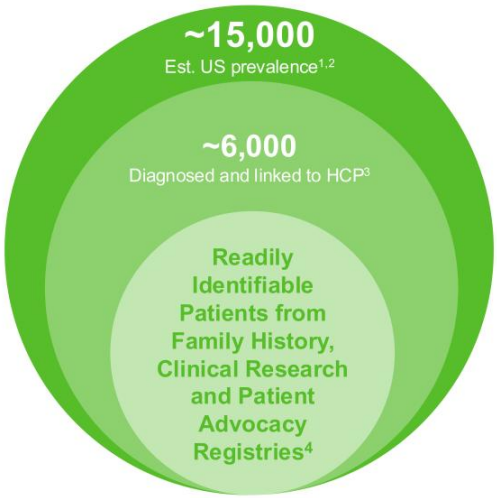
- Efficacy data from Studies 201, 206 and RWE
- 53% risk reduction in falls

Strong Safety/Tolerability Profile of Troriluzole

- Well-characterized safety profile of troriluzole assures a positive benefit-risk profile for SCA
- Studied in over 2,000 subjects across 8 years

KEY POINT NDA under Priority Review with anticipated approval and commercialization in 4Q 2025

SCA Represents a Significant Commercial Opportunity



- 6,000 diagnosed US patients
- No currently approved SCA treatments
- Availability of genetic testing and advent of approved treatment will facilitate diagnosis
- Engaged, connected SCA patient community
- Strong patient advocacy support
- KOLs, HCPs and key centers treating SCA have been identified

1. Ruano L, Melo C, Silva MC, Coutinho P. The global epidemiology of hereditary ataxia and spastic paraplegia: a systematic review of prevalence studies. Neuroepidemiology. 2014;42(3):174-83. 2. National Ataxia Foundation Website: https://www.ataxia.org/what-is-ataxia/, https://www.ataxia.org/neurologists-and-specialty-clinics/ , https://www.ataxia.org/crc-sca/. 3. Source: Patients filtered from LAAD claims data between April 2016 – Mar. 2021 purchased from IQVIA. 4. Data on File based on claims data purchased from IQVIA.

Existing SCA Market Dynamics Creates Opportunities for VYGLXIA® and Biohaven

VYGLXIA®

SCA MARKET DYNAMIC	VYGLXIA OPPORTUNITY
No approved therapies for SCA and no therapies in pivotal development	Establish VYGLXIA as the Standard of Care in SCA
SCA disease severity and ongoing progression	• VYGLXIA value proposition • Urgency to treat with need for chronic utilization
Autosomal dominant, inherited disease	Genetic testing programs to identify SCA patients and confirm diagnosis and map pattern of inheritance
Strong KOL and patient advocacy	• Motivated customers to drive early adoption of VYGLXIA • Support reimbursement
Centralized care in Centers of Excellence	Nimble, focused commercial footprint to launch VYGLXIA

15,000
Est. US Prevalence[1]

~6,000
Diagnosed and linked to HCP[2]

1. Ruano L, et al. *Neuroepidemiology*. 2014;42(3):174-83. 2. Data on File based on claims data purchased from IQVIA.

KEY POINT

- **VYGLXIA® name conditionally accepted by the FDA pending product approval**
- VYGLXIA® would be the first and only approved treatment for SCA

Strategic Imperatives to Drive Successful Launch in SCA

VYGLXIA®

SCA Launch Priorities

01 Identify patients, drive early diagnosis

02 Establish VYGLXIA® as SOC in SCA

03 Create access and reimbursement

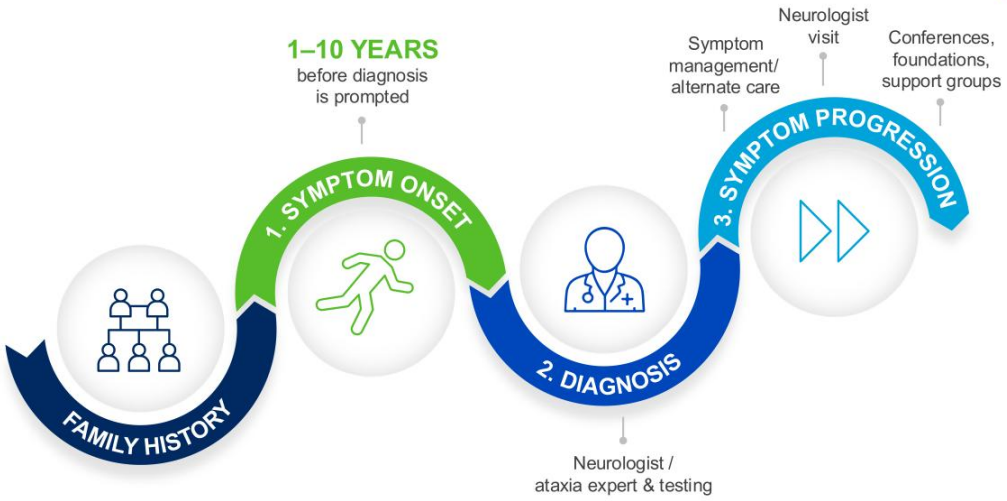
04 Ensure ongoing treatment

KEY POINT

SCA launch relies on identifying patients and driving diagnosis

Driving Patient Identification and Diagnosis Are Critical to Launch Success and Long-Term Growth

VYGLXIA®



Source: SCA Patient Journey Market Research. Conducted at NAF 2017 by Burke Institute. Updated: April 5, 2017

SCA Centralized Treatment Allows for Targeted and Efficient Commercialization Plan

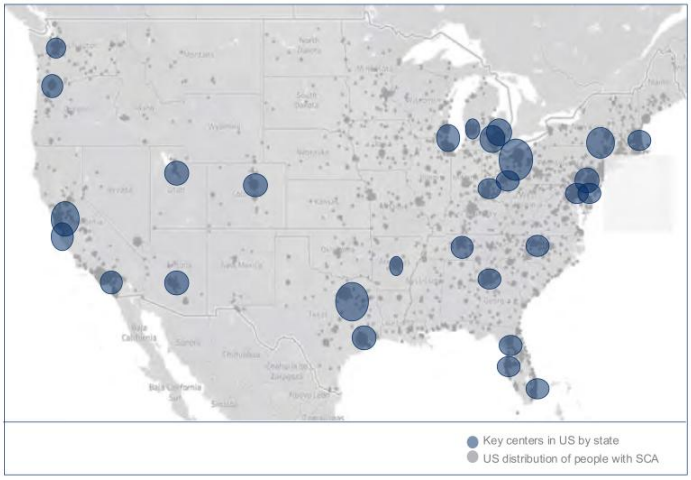
VYGLXIA®

SCA treatment at key centers

121 KOLs, 22 NAF Ataxia Centers of Excellence and 73 additional Movement Disorder and Ataxia Centers have been identified and manage many patients[1,2]

Experienced, efficient commercial team

- Proven track record in successful rare disease launches
- Planning to deploy dedicated SCA FTEs to drive a focused and rapid US launch



1. National Ataxia Foundation. Accessed May 12, 2025. https://www.ataxia.org/neurologists-and-specialty-clinics/. 2. Data on File based on claims data purchased from IQVIA.

VYGLXIA® in SCA Represents a Significant Commercial Opportunity

Readily identifiable patients will drive initial uptake

- High-volume ataxia experts and people with SCA interested or involved in clinical research programs/registries
- People already diagnosed with SCA and their families

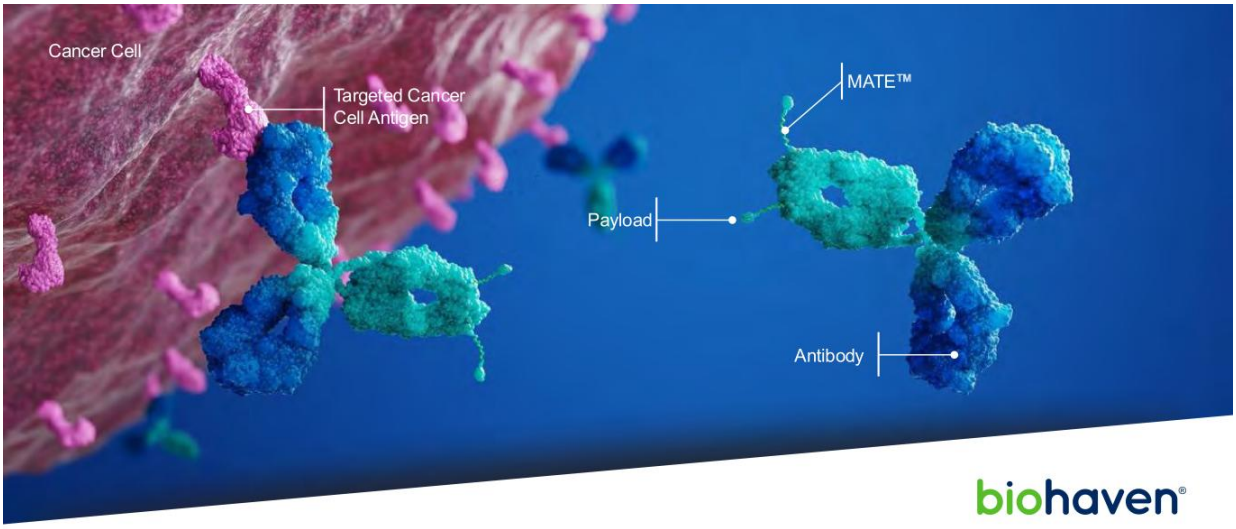
Biohaven's HUB will facilitate access to VYGLXIA®

- HUB will be a central point of contact for the SCA community
- Strive to ensure all SCA patients will have access to VYGLXIA®

Patient ID and early diagnosis fuel long-term growth

- Focused initiatives that drive patient ID and early diagnosis strategy are critical
- Targeted commercialization plan includes experienced rare disease leaders

KEY POINT If approved, the Biohaven team will be prepared to serve people living with SCA

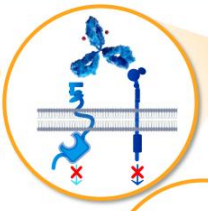


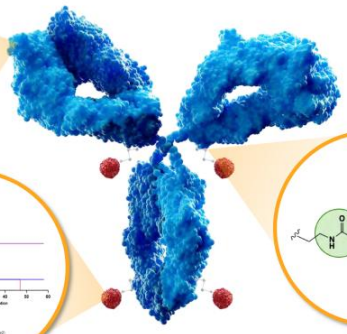
Oncology: Next-Generation ADCs

Biohaven's Innovative Technologies Modernizing Next-Gen ADCs

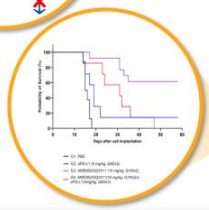
ONCOLOGY

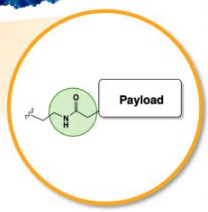
NOVEL AND BISPECIFIC mAbs
Validated and emerging targets





TOPOIX PROPRIETARY PAYLOAD
PD-1/PD-L1 synergy
Broad target exclusivity





MODERN ADC TECHNOLOGY, FLEXIBLE PLATFORM

- ✓ Site-specific
- ✓ Irreversible
- ✓ Single-Step
- ✓ Native mAbs
- ✓ Multiple payload class

STRATEGIC COLLABORATIONS	Merus	REGENERON	Yale	GeneQuantum Healthcare 启德医药
EXPANDING CLINICAL STAGE ASSETS	**BHV-1510** (Trop2 TopoIx) Phase 1 mono and anti-PD-1 combo	**BHV-1530** (FGFR3 TopoIx) Phase 1	**BHV-1500** (CD30 MMAE) IND planned 2H 2026	**Merus** (undisclosed)

BHV-1510 Is a Highly Differentiated Trop2 ADC

- Site-specific, highly stable conjugation-linker
- TopoIx payload ideally positions for fast-to-market strategy with anti-PD-1 combo
- Remains competitive as fast-follower

Emerging Clinical Data Shows Predicted Profile and Potential of Proprietary TopoIx Payload

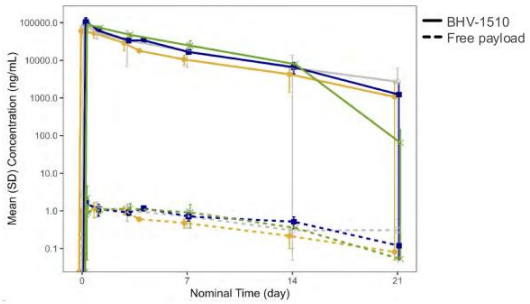
- Clinical activity demonstrated as monotherapy and in combination with anti-PD-1 cemiplimab
- No interstitial lung disease (ILD) with TopoIx observed in early cohorts
- Target exclusivity of TopoIx payload for up to 18 ADCs

BHV-1510: Favorable PK and Safety Profile With Very Low Free Payload

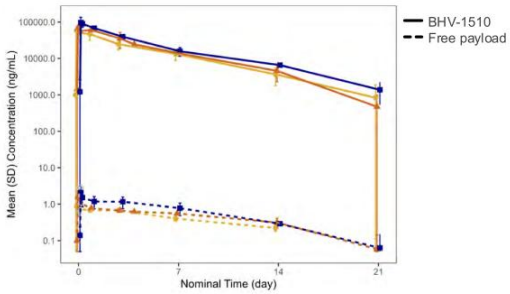
ONCOLOGY

- Mean serum exposure of BHV-1510 increased with increasing doses
- Very low levels of serum free payload, demonstrating high ADC stability

ADC and Payload, Semi-Log Scale: Cycle 1



ADC and Payload, Semi-Log Scale: Cycle 3



KEY POINT

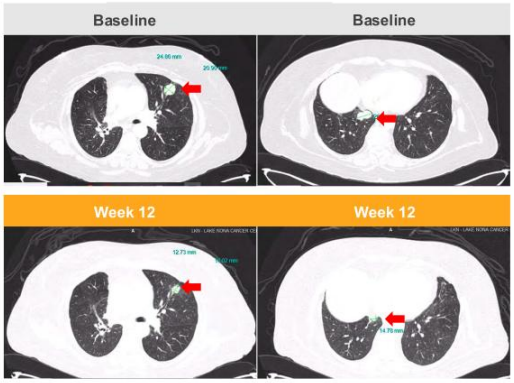
High ADC stability with payload to ADC ratio <1%

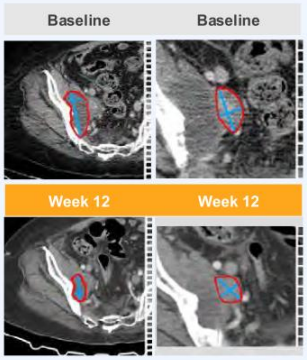
Monotherapy Shows PRs in Patients Failing Standard Therapies

ONCOLOGY

Case: 72 y/o with EGFRwt NSCLC

Prior therapies include carboplatin and paclitaxel, pembrolizumab then pemetrexed and carboplatin





Case: 60 y/o female with endometrial cancer

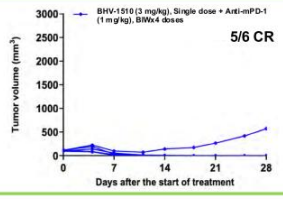
Prior therapies included dostarlimab, carboplatin and paclitaxel

BHV-1510 Combination With Anti-PD-1

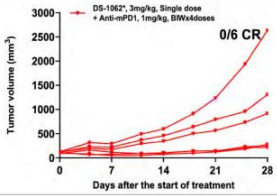
BHV-1510+Anti-PD-1: Syngeneic Models Suggest Superior to DS-1062

ONCOLOGY

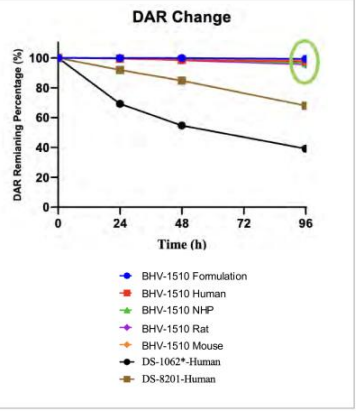
BHV-1510 + Anti-PD-1



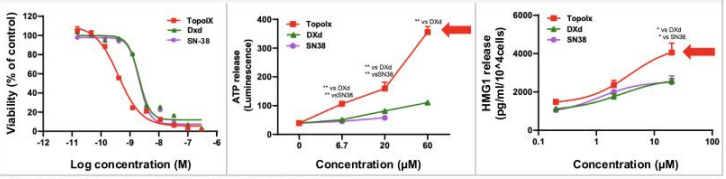
DS-1062 + Anti-PD-1



Highly Stable Linker vs DS-1062



Superior Immunogenic Cell Death with TopoIx Compared to Other Payloads (DXd and SN-38)



DS-1062, datopotamab deruxtecan; AACR 2023 Annual Meeting, abstract 1549.

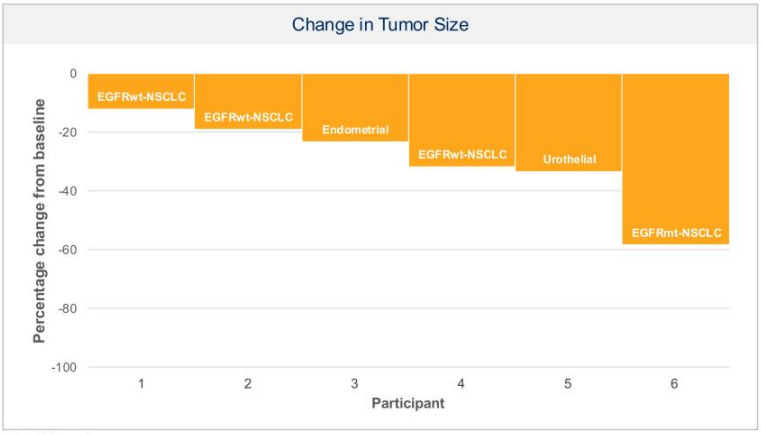
KEY POINTS

- Preclinical profile of BHV-1510 positions it for superiority in ADC therapy with anti-PD-1
- Landscape open for Trop2 combinations with safer more efficacious ADC

BHV-1510 + Cemiplimab: Preliminary Activity Observed With Tumor Reduction Seen in First 6 Patients Treated

ONCOLOGY

- Encouraging preliminary efficacy with tumor reductions seen on first scan for all patients
- Partial responses starting at lowest dose tested
- Clinical activity demonstrated in patients failing standard of care therapies. Majority with prior anti PD1/PL1 agents
- Responses seen in patients with brain metastasis
- Combination well tolerated; no DLTs or ILD in initial cohorts



EGFR: Epidermal Growth Factor Receptor, **WT**: Wild type, **MT**: Mutant, **NSCLC**: Non-Small Cell Lung Cancer

KEY POINTS

- Early data suggests synergy with an anti-PD1, with a favorable safety profile
- Potential to move to early lines of therapy in tumors like urothelial, NSCLC and endometrial carcinoma

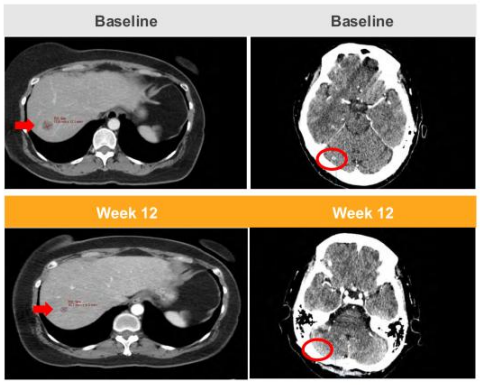
Partial Response in Patients Failing SOC Therapy Including Targeted Therapy and Immunotherapy

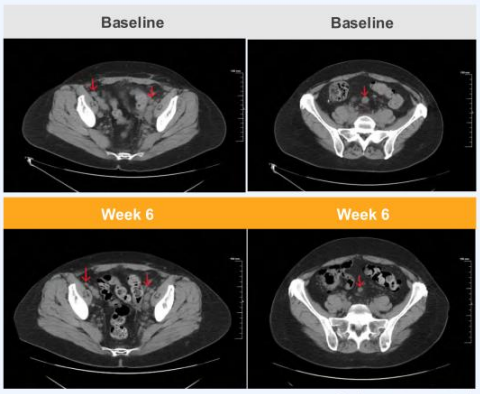
ONCOLOGY

Case: 57 y/o female with EGFRmt NSCLC and Brain Metastasis

Confirmed PR (58% reduction) at 12 weeks after 1510+cemiplimab combo

- Prior therapies included cisplatin and pemetrexed chemotherapy
- Failed two EGFR targeting therapies including EGFR-MET bispecific amivantamab-vmjw and investigational EGFR-HER2 inhibitor





Red arrows: tumor metastasis

Case: 53 y/o with urothelial cancer

PR (33% reduction) at 6 weeks after 1510+cemiplimab combo

- Prior therapies included cisplatin and gemcitabine chemotherapy
- Enfortumab Vedotin and pembrolizumab for metastatic disease.

BHV-1510: Tumor Reduction Seen in Difficult to Treat Tumors, in Patients Progressing on Standard and Approved Therapies

ONCOLOGY

- Clinical activity seen across doses starting at the lowest dose
- As monotherapy partial response seen in tumor types (SCLC, Endometrial, NSCLC) in patients that are heavily pretreated and have progressed on standard of care therapies
- Patients on treatment with durable clinical benefit, several at 6 months and beyond
- Tumor reductions seen in first 6/6 evaluable patients treated with cemiplimab combination; PRs in urothelial and NSCLC patients
- Favorable preliminary safety profile
  - No payload-associated ILD, low GI toxicity like diarrhea, and low hematological toxicity
  - Main toxicity observed is on-target Trop2 ADC class mucositis; an expected and manageable effect
  - Combination with cemiplimab well tolerated with no DLTs to date in initial cohorts

Prelim data from ongoing study. Data cut off May 2, 2025

KEY POINTS

Preliminary efficacy and tolerability of BHV-1510, incorporating the novel TopoIx payload and highly stable linker, indicate potential for use in earlier treatment settings across various cancers

BHV-1530

Novel FGFR3 mAb With Proprietary Topolx Payload

- Only FGFR3 directed ADC in clinic
- Site-specific conjugation with favorable nonclinical tox profile

FGFR3 Is a Validated Target With Limited Competition

- No current FGFR3 ADCs approved or in advanced development Core opportunity in FGFR3-altered metastatic urothelial cancer
  - Only 1 Tyrosine Kinase Inhibitor approved
  - TKI toxicity due to Pan FGFR inhibition
- Potential extension into other FGFR3-driven solid tumors

Synergistic Efficacy With Checkpoint Inhibitors *In Vivo*

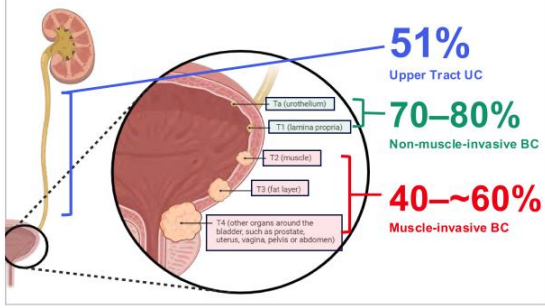
Anti-PD-L1 combination showed synergy similar to BHV-1510

FGFR3 Is a Promising Therapeutic Target in Several Tumors

ONCOLOGY

FGFR3 Overexpression and Alteration in Urothelial Carcinoma

FGFR3 Overexpression and Alteration (%)



51%
Upper Tract UC

70–80%
Non-muscle-invasive BC

40–~60%
Muscle-invasive BC

HSCC, Head and Neck Squamous Cell Carcinoma.

~15% of HNSCC have FGFR3 mutations with overexpression noted in nearly half of oral and oropharyngeal cancers

FGFR3 expression in **38% of lung cancers** suggests substantial overexpression within these types

5% of endometrial cancers have FGFR3 alterations, potentially indicating more widespread overexpression

4% of cervical cancer cases exhibit the FGFR3-TACC3 fusion, which is associated with higher FGFR3 expression

~ 8% of glioblastomas show a FGFR3-TACC3 fusion, commonly linked to increased levels of FGFR3 protein

KEY POINT

ADC and CPI combinations are emerging as a powerful strategy in urothelial cancer—offering the potential to drive deeper, more durable responses and shift the standard of care

BHV-1530: Potential to Address Unmet Need in Metastatic Urothelial Cancer (mUC) and other FGFR3-Driven Tumors

ONCOLOGY

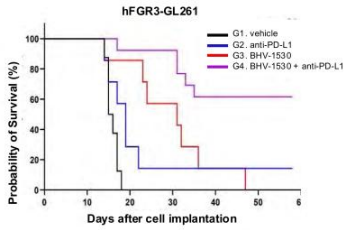
FGFR3 overexpression, mutation, or **fusion** leads to excessive pathway activation and increased **tumorigenicity**

FGFR3 mutation/fusion ~20% mUC

FGFR3 overexpression ~35% mUC

- 62K new mUC cases, 14K deaths/year in US (2023)
- Multiple opportunities for BHV-1530 across therapy lines
  - Synergistic CPI combinations in FGFR3+ biomarker-selected 1L
  - Limited efficacy of current 2L options
- Several tumor types beyond mUC also driven by FGFR3

BHV-1530 shows **synergistic activity** *in vivo* with anti-PD-L1 combination



Group	% Increased Life Span (ILS)	Median Survival (days)
G1	-	15
G2	27%	19
G3	107%	31
G4	>300%	>63

BHV-1530: Phase 1 Study in Advanced Tumors

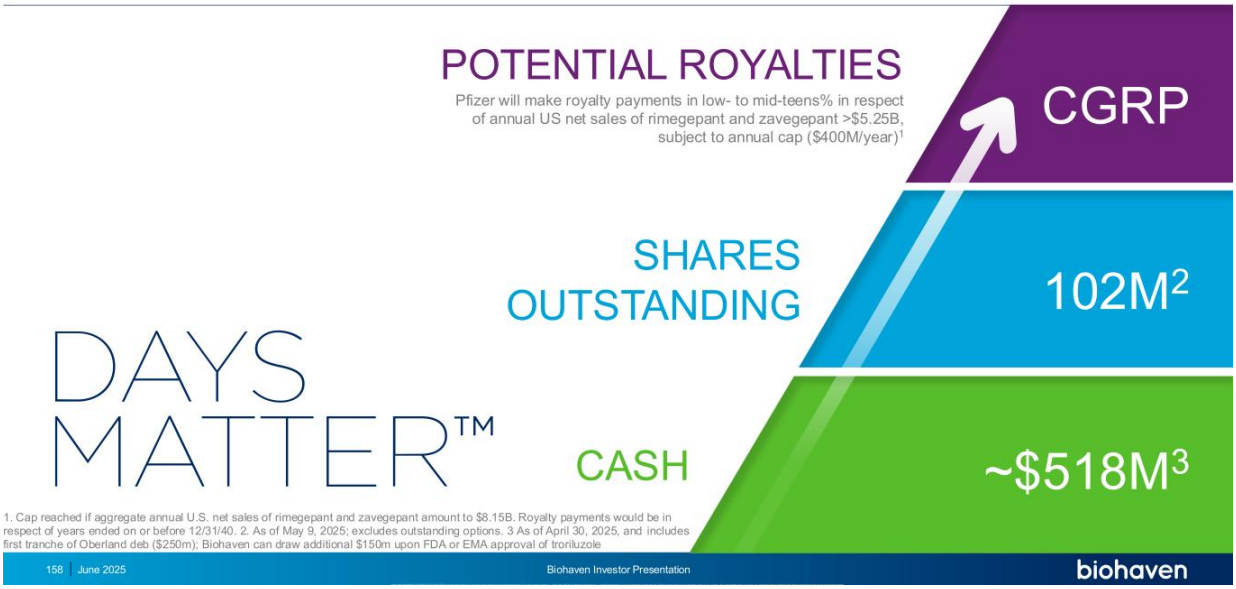
ONCOLOGY

DESIGN	Open label, dose escalation (Ph1)
POPULATION	Advanced tumors having failed SOC therapy
SAMPLE SIZE	95 patients
TREATMENT	BHV-1530
TREATMENT DURATION	Until disease progression or toxicity
KEY ENDPOINTS	Safety and tolerability, ORR, PFS, PK and ADA

ORR, Overall Response Rate; **PFS,** Progression Free Survival; **ADA,** Antidrug Antibody; **BOIN**, Bayesian Optimal Interval; **RD**, Recommended Dose.

BREAKING NEWS First patient dosed in April 2025

Liquidity Update



1. Cap reached if aggregate annual U.S. net sales of rimegepant and zavegepant amount to $8.15B. Royalty payments would be in respect of years ended on or before 12/31/40. 2. As of May 9, 2025; excludes outstanding options. 3 As of April 30, 2025, and includes first tranche of Oberland deb ($250m); Biohaven can draw additional $150m upon FDA or EMA approval of troriluzole

Key Milestones Anticipated in 2025–2026

Milestone achieved

			1H 2025	2H 2025	2026
GLUTAMATE	Troriluzole | BHV 4157	Spinocerebellar Ataxia	Completed Mid-cycle Review	PDUFA/Launch (if Approved)	
		Obsessive-Compulsive Disorder	Phase 3 Study #1 Topline		Phase 3 Study #2 Topline
MYOSTATIN	Taldefgrobep Alfa | BHV-2000	Spinal Muscular Atrophy	FDA Meeting		
		Obesity		Initiate Phase 2	
ION CHANNEL	Kv7 Activator | BHV-7000	Focal Epilepsy			Phase 3 Focal #1 Topline
		Major Depressive Disorder		Pivotal Topline	
	TRPM3 Antagonist | BHV-2100	Pain Disorders	Migraine POC Negative		
INFLAMMATION & IMMUNOLOGY	TYK2/JAK1 Inhibitor | BHV-8000 (brain-penetrant)	Parkinson's Disease (PD)	Initiated Phase 2/3		
	IgG Degrader | BHV-1300 | BHV-1310	Common Disease (Graves', RA)	Completed Phase 1	Initiate Pivotal Graves Study	
		Rare Disease (Myasthenia Gravis)	Initiate Phase 1		Initiate MG study
	Gd-IgA1 Degrader | BHV-1400	IgA Nephropathy	Completed Phase 1		Initiate pivotal IgAN study
	β1AR AAb Degrader | BHV-1600	Peripartum Cardiomyopathy		Complete Phase 1	Initiate pivotal PPCM study
ONCOLOGY	Trop2 ADC +/- PD1 | BHV-1510	Advanced or Metastatic Epithelial Tumors		Phase 1 Interim Data	
	FGFR3 ADC | BHV-1530	Urothelial Cancer & Other Tumors	Initiated Phase 1		
	CD30 ADC | BHV-1500	Hodgkin Lymphoma	Regulatory Interaction		
	Undisclosed Targets	*Merus and GeneQuantum Collaborations*	Advance New ADCs		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