biohaven®

BHVN
LISTED
NYSE

# DAYS MATTER™

## Corporate Presentation




November 2026

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# 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 (including those for our taldefgrobep alfa, opakalim, BHV-2100, BHV-8000, BHV-8100, BHV-1300, BHV-1310, BHV-1400, BHV-1510, BHV-1530 and BHV-1600 development programs), the timing of and the availability of data from our clinical trials, the timing of 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-1955, BHV-8200, BHV-2110, BHV-1490, BHV-1420, BHV-1440, BHV-6500 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 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 resubmission of our new drug application for troriluzole for SCA; the timing and outcome of expected regulatory filings Biohaven's compliance with applicable U.S. regulatory requirements; the potential commercialization of Biohaven's product candidates; 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.

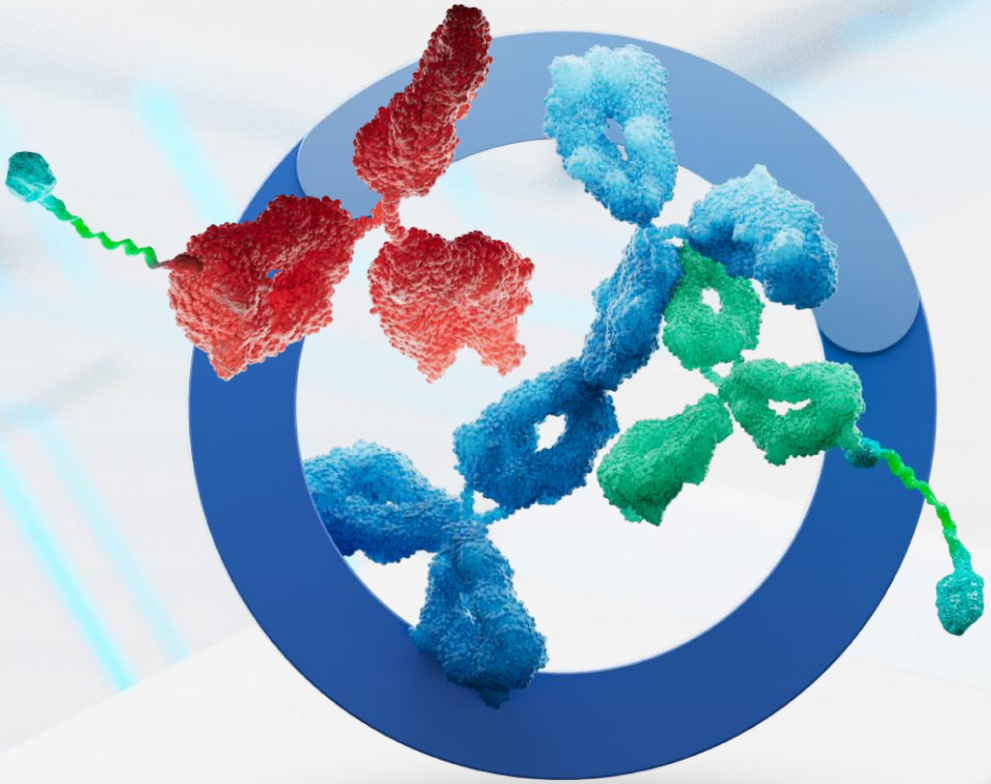
| | | | | PRECLINICAL | PHASE 1 | PHASE 1B/PHASE 2 | PHASE 3 | MARKET |
|---|---|---|---|---|---|---|---|---|
| GLUTAMATE | Troriluzole | BHV-4157 | Spinocerebellar Ataxia | | | | | |
| INFLAMMATION & IMMUNOLOGY | IgG Degrader | BHV-1300 | Common Disease (Graves', RA) | | | | | |
| | Gd-IgA1 Degrader | BHV-1400 | IgA Nephropathy | | | | | |
| | β1AR AAb Degrader | BHV-1600 | Peripartum Cardiomyopathy | | | | | |
| | TYK2/JAK1 Inhibitor (brain-penetrant) | BHV-8000 | Parkinson's Disease | | | | | |
| ION CHANNEL | Kv7 Activator | Opakalim | Focal Epilepsy | | | | | |
| | TRPM3 Antagonist | BHV-2100 | Pain Disorders | | | | | |
| MYOSTATIN ACTIVIN | Taldefgrobep Alfa | BHV-2000 | Obesity | | | | | |
| ONCOLOGY | Trop2 ADC +/- PD-1 | BHV-1510 | Advanced or Metastatic Epithelial Tumors | | | | | |
| | FGFR3 ADC | BHV-1530 | Urothelial Cancer and Other Tumors | | | | | |
| PRE-CLINICAL DEGRADERS | IgG Degrader | BHV-1310 | Rare Disease (Myasthenia Gravis) | | | | | |
| | | BHV-1320 | Immune-Mediated Disease | | | | | |
| | IgG4 Degrader | BHV-1450 | Pemphigus, MuSK MG, LGI-1 Encephalitis | | | | | |
| | PLA2R AAb Degrader | BHV-1420 | Membranous Nephropathy | | | | | |
| | TSHR AAb Degrader | BHV-1440 | Graves' Disease and TED | | | | | |
| | Proinsulin AAb Degrader | BHV-6500 | Type 1 Diabetes | | | | | |
| | IgM Degrader | BHV-1490 | IgM Neuropathy, Waldenstrom's | | | | | |
| EMERGING TARGETS | PKM2 Activator | BHV-8100 | Neurodegenerative Diseases | | | | | |
| | Intranasal Oxytocin | BHV-1955 | Tinnitus | | | | | |

**AAb**, Autoantibody.

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# DEGRADERS: MoDE™ AND TRAP™

## Targeting the Root Cause of Autoimmune Disease



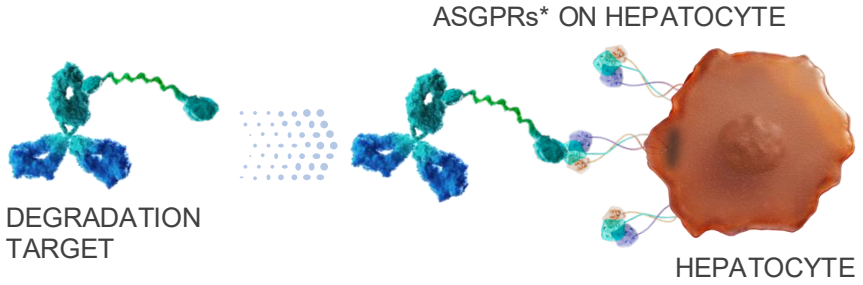
# Innovation in Protein Degradation: Harnessing Hepatic ASGPR for Efficient and Safe Removal of Circulating Pathogenic Targets

DEGRADERS

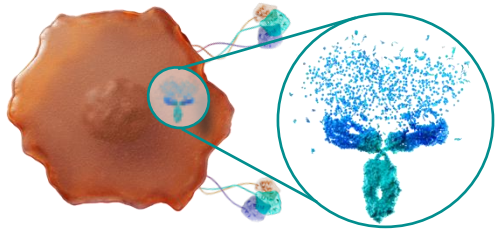
BIFUNCTIONAL
MoDE DEGRADERS



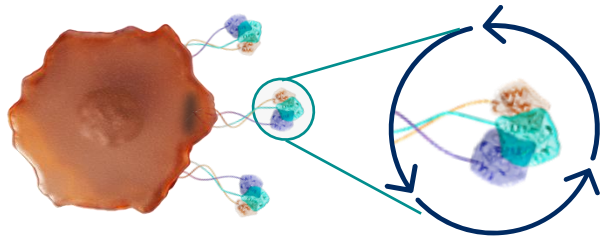

1. MoDE™ and TRAP™ degraders bind circulating target and efficiently deliver to ASGPR on hepatocytes




2. Internalized target is rapidly degraded in hepatic lysosomes



3. ASGPRs are rapidly recycled

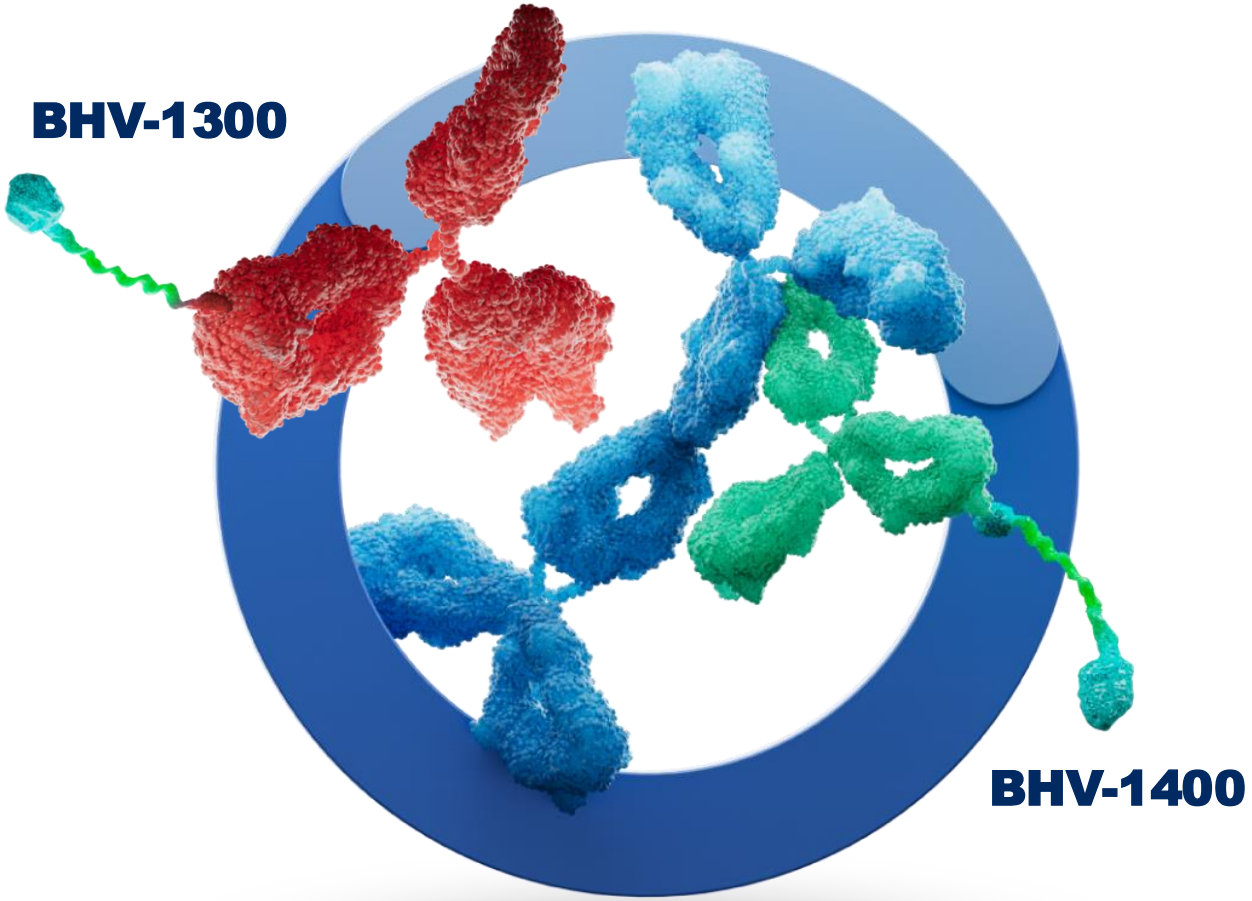


MoDE and TRAP optimized for patient-friendly **autoinjector delivery**

*Stylistic representation
**ASGPR**, asialoglycoprotein receptor

**MoDE™ and TRAP™**
DEGRADERS

# First Extracellular Protein Degraders in the Clinic Demonstrate Rapid and Robust Pharmacodynamic Effects and Compelling Safety in Nearly 200 Individuals Dosed




- Safe
- Well-tolerated
- Highly selective
- Deep and rapid lowering
- Patient outcomes
- Pivotal trials 2026

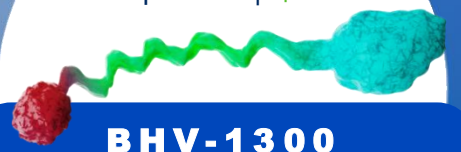
# MoDE™ and TRAP™ Degraders

**PIPELINE ASSETS**
**8+**
**BHV degrader programs**

**ADDRESSABLE PATIENTS**
**>2M**
**across lead programs (US)**

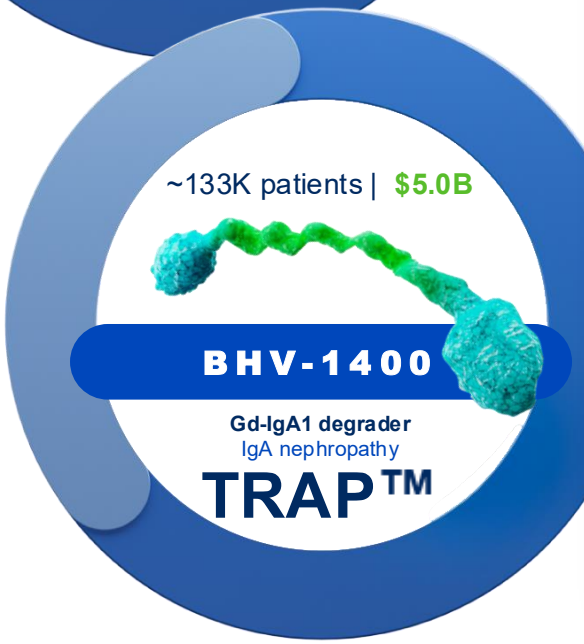
**PEAK SALES POTENTIAL**
**$30B+**
**combined gross sales**

## MoDE™
>1M patients | $7.6B



**BHV-1300**

**IgG degrader**
Graves' disease · RA
Sjögren's · Biologic failures







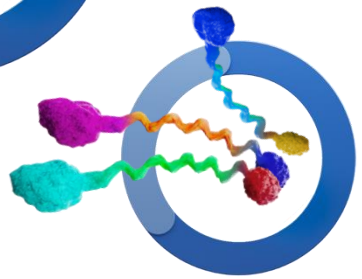
~400K patients
$11.1B
**IgG Degrader**
gMG
Systemic sclerosis
AE
ITP

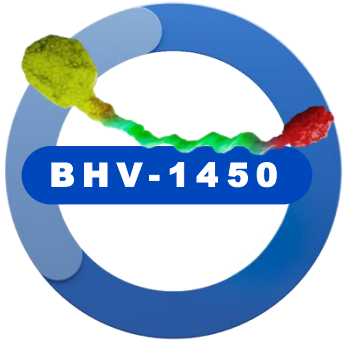



~42K patients
$1.7B
**IgM degrader**
Cold agglutinin disease
Anti-MAG
Waldenström's




~41K patients
$1.3B
**PLA2R AAb Degrader**
Membranous nephropathy (MN)






~92K patients
$2.6B
**IgG4 degrader**
Pemphigus vulgaris
MuSK MG
IgG4-RD
CIDP




**TSHR AAb degrader**
Graves' disease
Thyroid eye disease



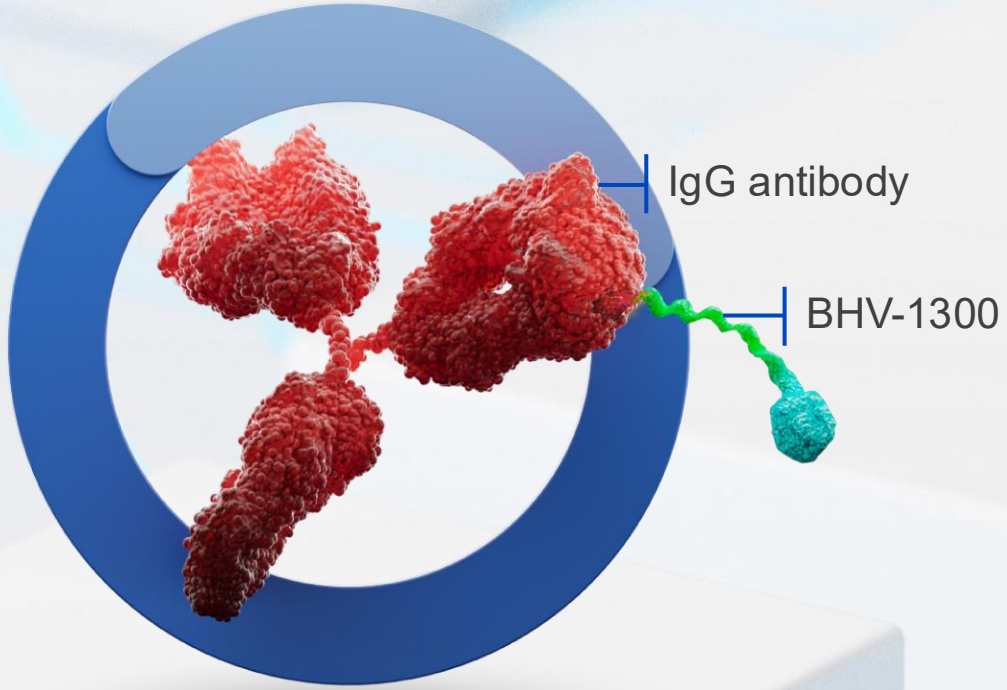

**Pro-Insulin/Insulin AAB degrader**

Addressable patients for each degrader based on internal assessment of potential patient population. Peak sales potential based on addressable patients for each degrader and internal assessment of gross revenue potential (prior to GTN adjustments or any adjustments associated with clinical risk), adjusted for estimated market share / market penetration.

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**IgG MoDE™ DEGRADER:**
**BHV-1300**

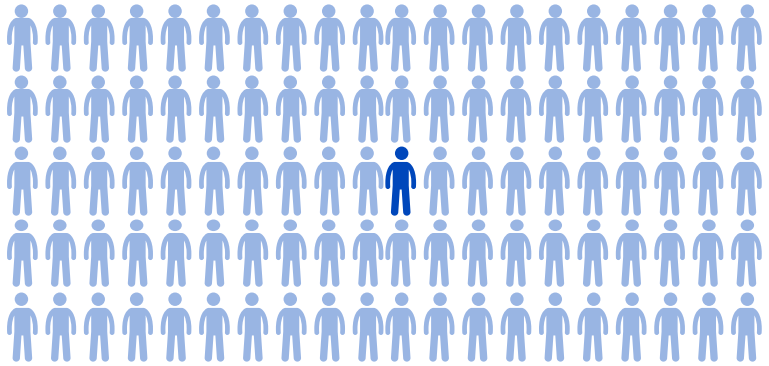
# Targeting the Root Cause of Graves' Disease



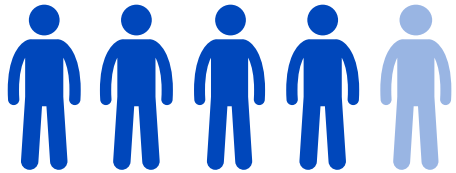
# Demographics: Graves' Disease Impacts 1% of the Global Population



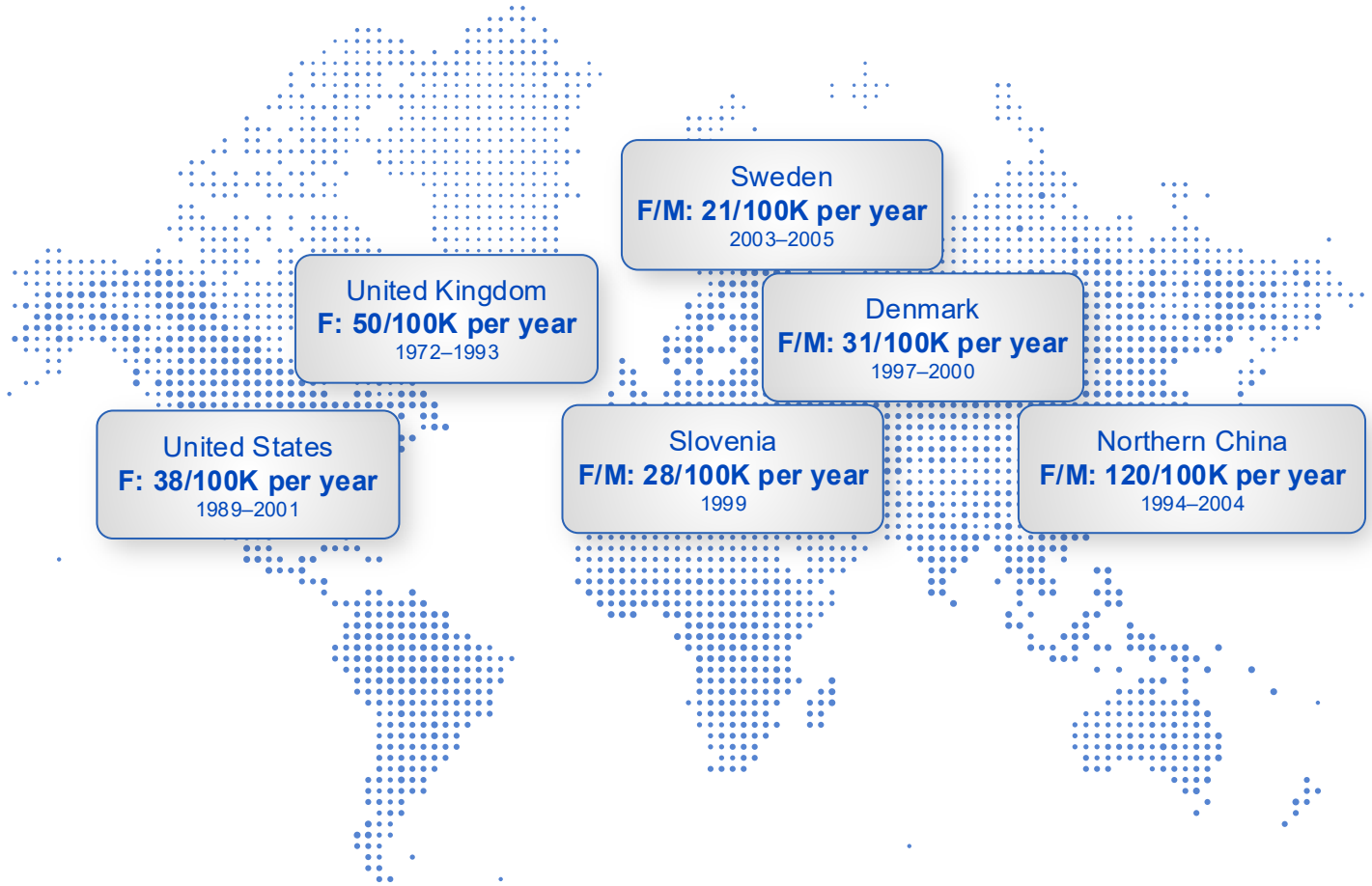
**Graves' disease affects nearly 1 in 100 Americans.**[1]



**About 4 out of 5 cases of hyperthyroidism in the United States are caused by Graves' disease.**[1]



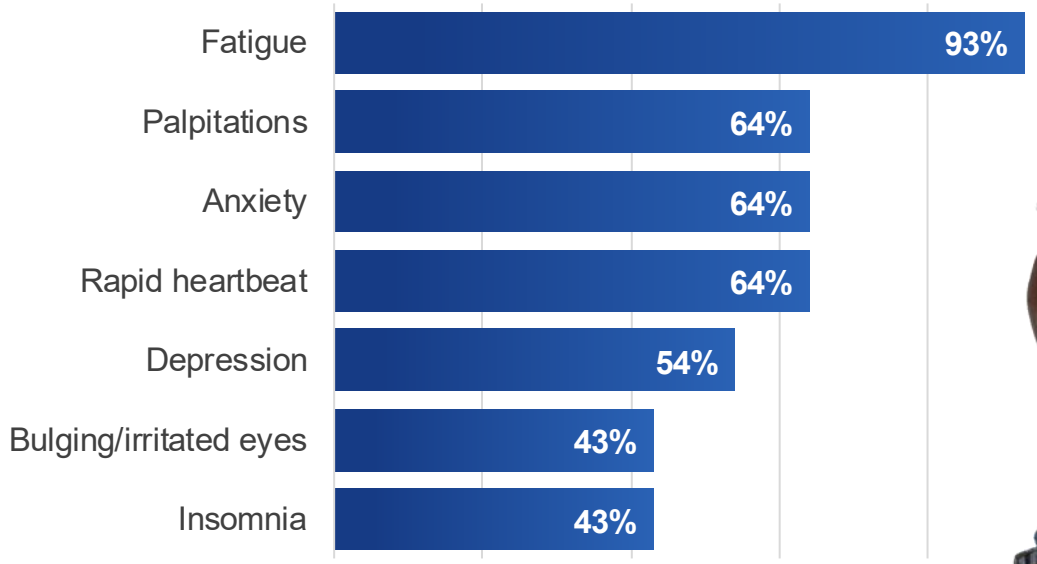
**Worldwide incidence of Graves' disease**[2]



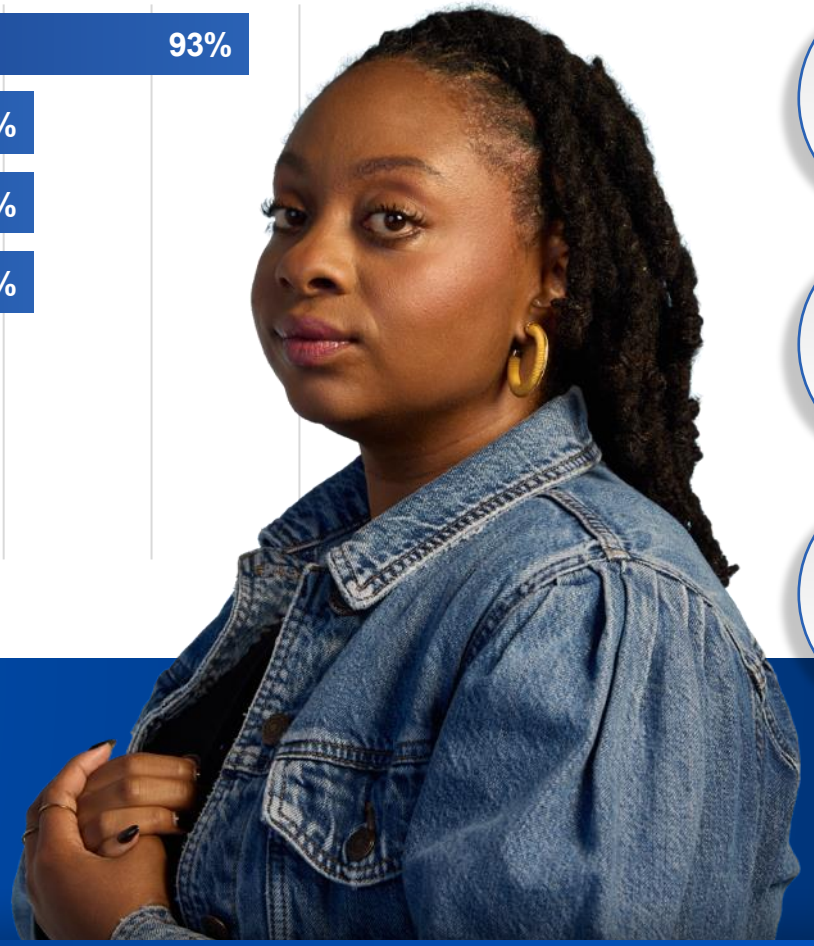

**1.** Akram. Journal of Surgical Research. 2020. **2.** McLeod. Endocrine, 2012.

# The Under-Recognized Burden of Graves' Disease[1]

DEGRADERS




1. Patient Burden in Graves' Disease: Results From a Mixed Methods Survey



**93%** report multiple symptoms (≥2)

**79%** experience 4+ symptoms

**72%** experience 5+ symptoms

**Even among biochemically well-controlled patients,**
**75% report recurring symptoms —**
fatigue, palpitations, anxiety, weight gain

Courtnay
Living with Graves' disease

KEY POINT

Current treatments achieve biochemical control but do not address the underlying antibody-driven disease, antibodies continue to circulate, targeting the thyroid, orbit, brain, and crossing the placenta

# Current Treatments for Graves' Disease

## CONSERVATIVE

1. **Use of thionamides**
   (ATDs, like methimazole, carbimazole, and propylthiouracil (PTU)
   *Aplastic anemia, agranulocytosis, peripheral neuritis, liver issues, secreted in breastmilk*
2. **Use of steroids**

## ABLATIVE

3. **Surgical removal of the thyroid**
   Lifelong T4 replacement, surgical complications
4. **Use of I-131 radiation treatment**
   Cannot use in pregnancy, TED worsens



# BHV-1300 MoDE™ Targets the Root Cause of Graves' Disease and TSHR Autoantibody-Driven Diseases

DEGRADERS

**EXCESS HORMONES**

T3 T4

**TSHR AUTOANTIBODY DRIVEN DISEASES**

- Thyroid eye disease
- Graves' embryopathy
- Thyroid dermopathy
- Graves' disease

**STABILIZED HORMONES**

T3 T4

BHV-1300

**BHV-1300 degrades disease-causing TSHR-IgG1 antibodies**

# Not an FcRn Inhibitor: Biohaven IgG MoDE™ Degrader Differentiates as a Novel MOA, Potential Paradigm-Shifting Therapy

DEGRADERS

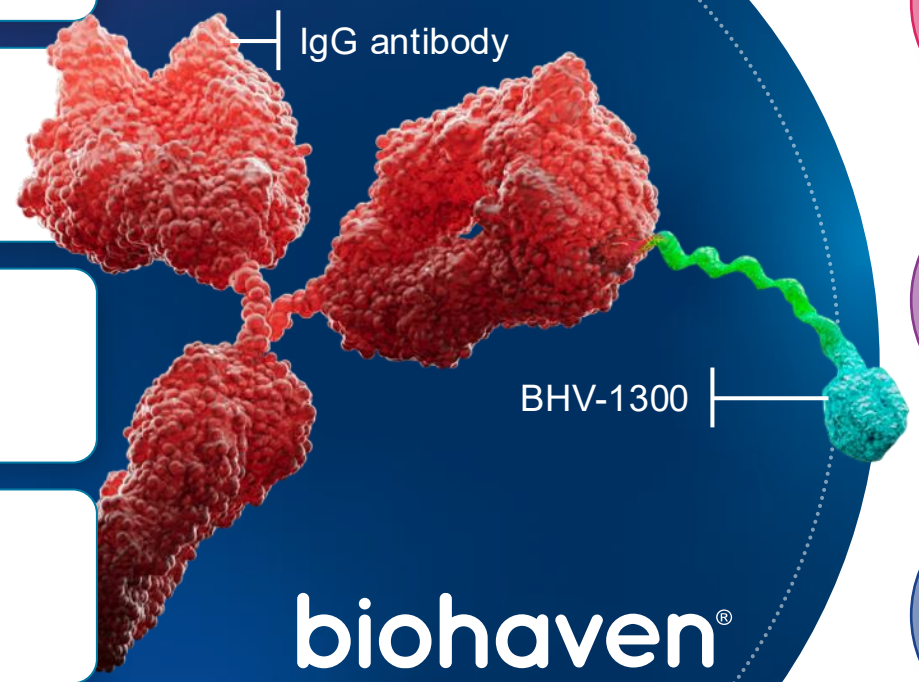
**83% IgG LOWERING**
BY DAY 18

**DID NOT INCREASE**
HEADACHES

**DID NOT INCREASE**
CHOLESTEROL

**SMALL MOLECULE**

**AUTOINJECTOR**
ADMINISTRATION IN PIVOTAL TRIALS







**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 at week 4 (VYVGART Hytrulo® in MG trial)[3] (average 75% in MAD)[4]
- Prefilled syringe
- Cyclical dosing can lead to symptom rebound

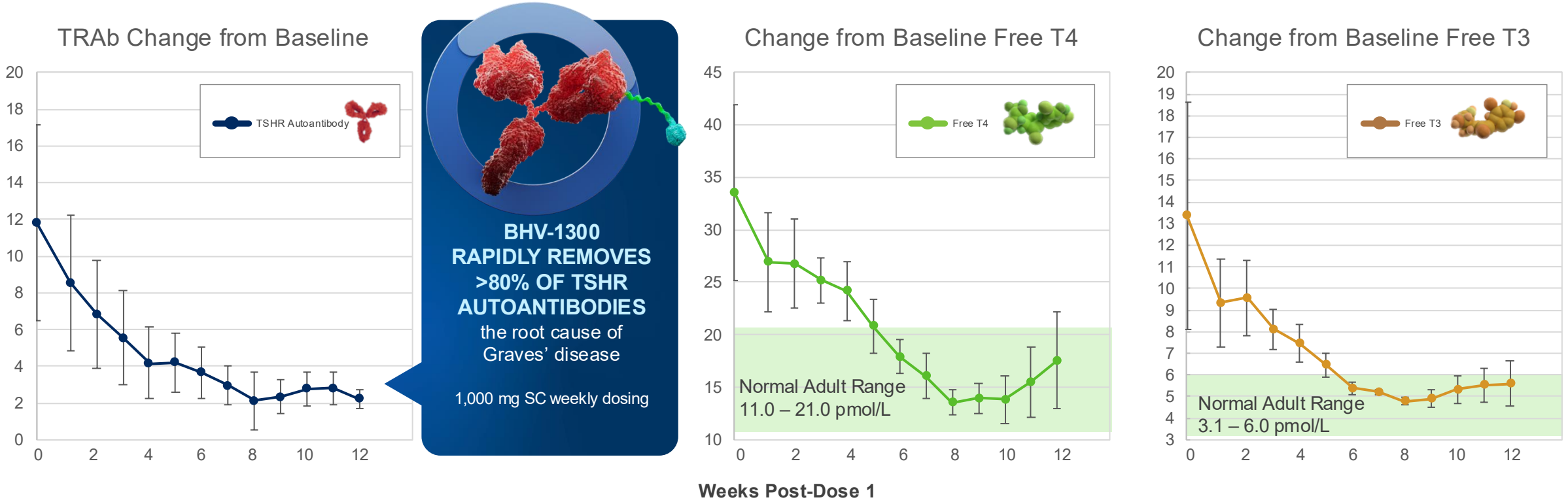
**Rystiggo® UCB**

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

**1.** Median of the maximal total IgG % change from baseline **2.** 84% IgG lowering (twice the labeled frequency) in Phase 1; Ling. Clin Pharmacol Ther. 2019. **3.** Howard, Jr. ADAPT (SC) Data – 2024; **4.** Ulrichts. J. Clin. Invest. 2018. **5.** MAD data unavailable. MG Data from Bril, Lancet Neurology. 2023 – MyCarinG study.

# BHV-1300 MoDE™ Deeply Removes Disease-Driving TSHR Autoantibody and Normalizes Thyroid Hormones

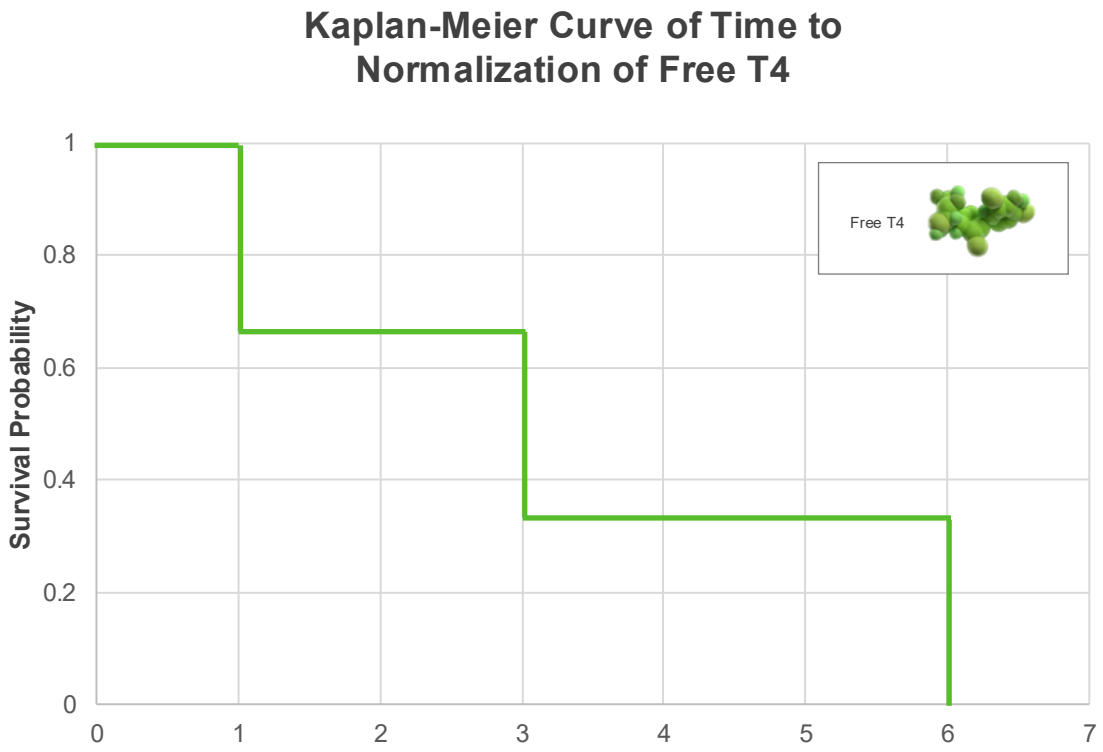
DEGRADERS

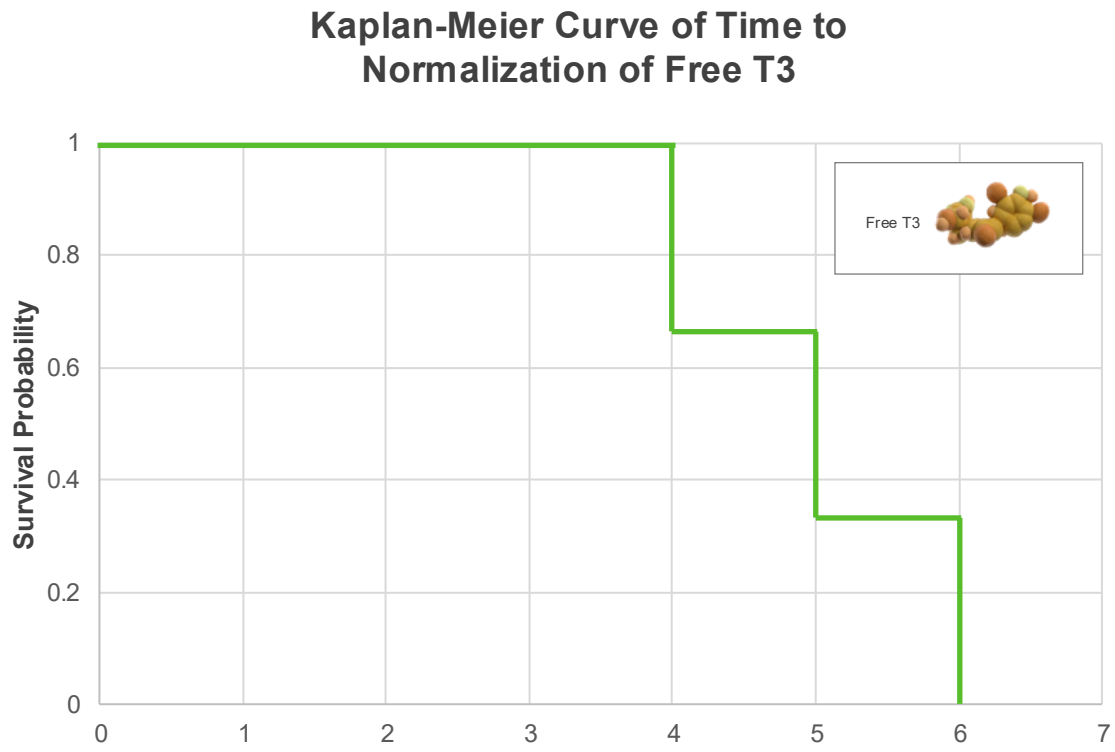



Preliminary data from ongoing study, analysis conducted May 22, 2026. Data represents mean and standard error concentration of TRAb, Free T4, and Free T3 in the three consecutive overt hyperthyroid participants with Graves' disease. Participants were dosed with BHV-1300 SC 1000 mg weekly for 12 weeks (n=2) or BHV-1300 1000 mg SC weekly for 4 weeks followed by 500 mg SC weekly for 8 weeks. Values of TRAb below the limit of quantification (LOQ) were set to LOQ/2.

# BHV-1300 Rapidly Normalizes Thyroid Hormones in Patients With Graves' Hyperthyroidism

DEGRADERS




**Weeks Post-Dose 1**

Preliminary data from ongoing study, analysis conducted April 8, 2026. Graphs represent time to normalization of Free T3 and Free T4 in participants (n=3) with Graves' disease and overt hyperthyroidism at baseline administered BHV-1300 1,000 mg SC weekly for 12 weeks (n=2) or BHV-1300 1,000 mg SC weekly for 4 weeks followed by 500 mg SC weekly for 8 weeks (n=1).

**KEY POINT**

In hyperthyroid patients receiving BHV-1300 1,000 mg SC weekly, Free T4 normalized within an average of 3 weeks and Free T3 normalized within an average of 5 weeks

# Lead MoDE™ Degrader, BHV-1300 Enters Phase 3






## KEY STUDY DETAILS

**Study Design:** Randomized, double-blind, placebo-controlled trial

**Population:** Male and female adults with Graves' disease

**Endpoints:** Normal T3, T4 and TSH off ATD at week 26

**ATD**, antithyroid drugs

**KEY POINT**

BHV-1300 pivotal trial in Graves' disease initiated



biohaven®

ONO PHARMA

COLLABORATION

## Translating scientific breakthroughs into medicines that matter

- Validates Biohaven's extracellular protein degrader technology
- Leverages Ono's immunology and endocrinology heritage and commercial leadership in the region

Strongest and fastest path to patients in Japan and key Asian markets

Up to

**$100M NEAR-TERM**

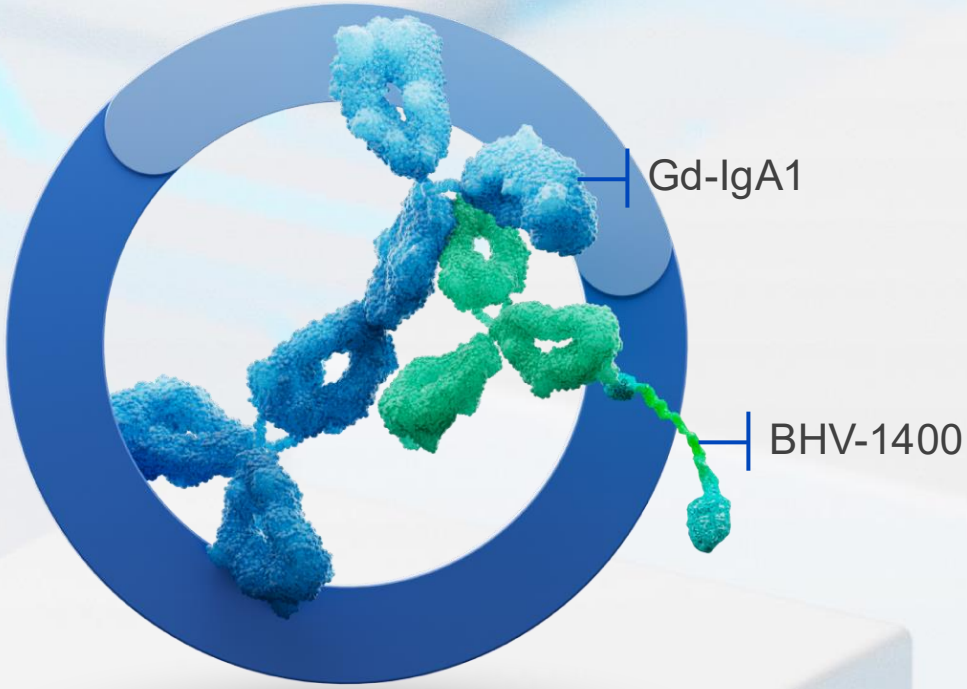
~20%

**ROYALTY**

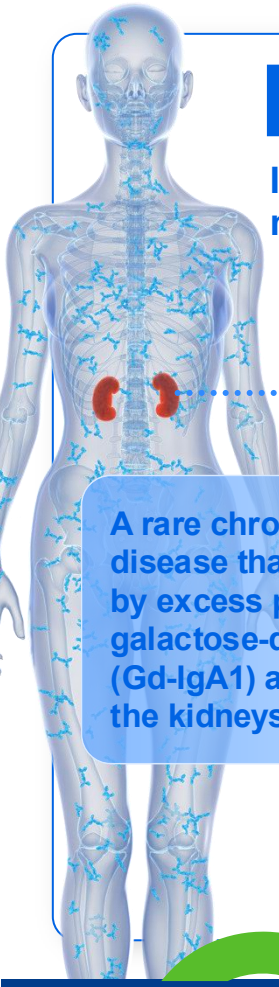
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## Gd-IgA1 TRAP™ DEGRADER: BHV-1400

# Targeting the Root Cause of IgA Nephropathy



# By the Time IgAN Is Diagnosed, Kidney Function Is Already Significantly Compromised



## IgAN

**IgA nephropathy**

**A rare chronic kidney disease that is caused by excess production of galactose-deficient IgA1 (Gd-IgA1) antibodies in the kidneys**

IgAN predominantly affects people in their **MOST PRODUCTIVE YEARS OF LIFE**

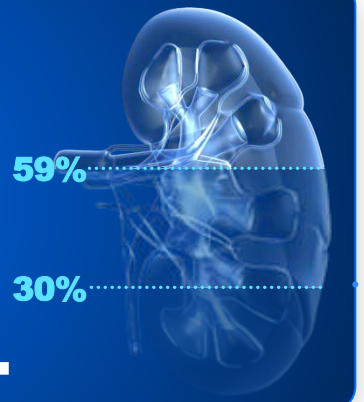
| **20s** | **30s** |
|---|---|
| MEDIAN AGE AT DIAGNOSIS[1] | MOST COMMON AGE RANGE[1] |

## CKD STAGE 3

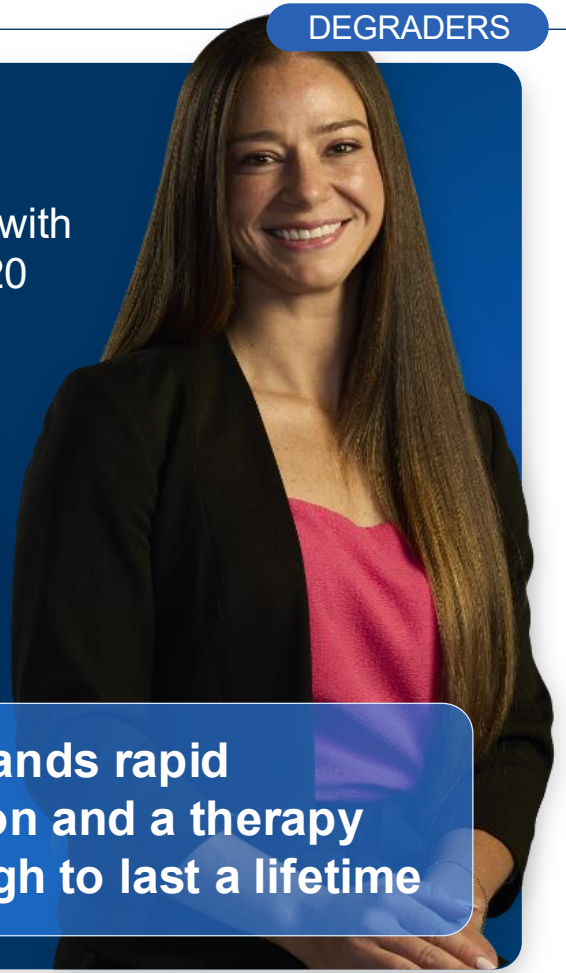
MOST COMMON AT DIAGNOSIS

**At diagnosis, kidney function is already reduced to**

**30–59% OF NORMAL**




DEGRADERS



**Camille**

Diagnosed with IgAN in 2020 at age 25

**IgAN demands rapid intervention and a therapy safe enough to last a lifetime**

1. Pitcher. Kidney Int. 2023.

TREATMENT GOAL

**Every month matters** — rapid intervention to preserve kidney function and limited remaining nephrons

# Guidelines Focus on Removal of Pathogenic Gd-IgA1

KDIGO

DEGRADERS

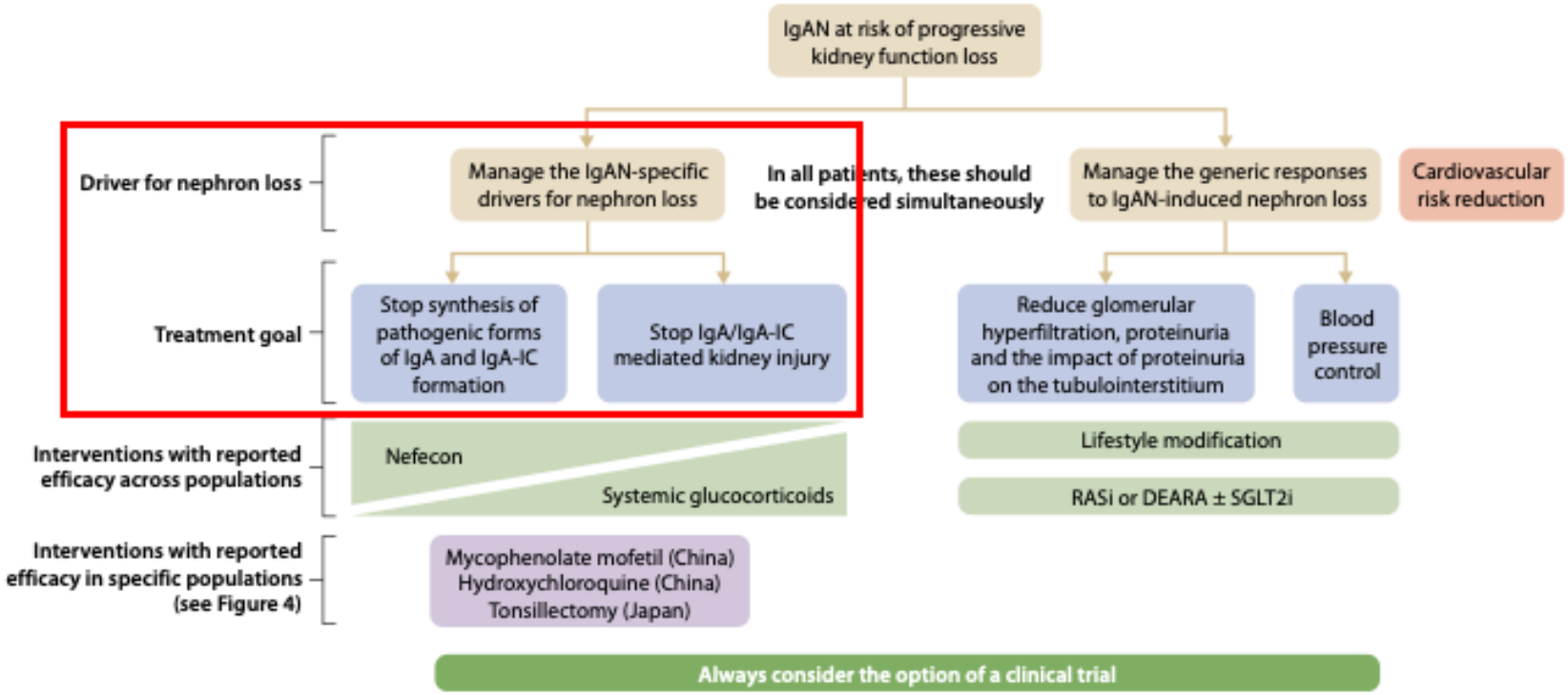
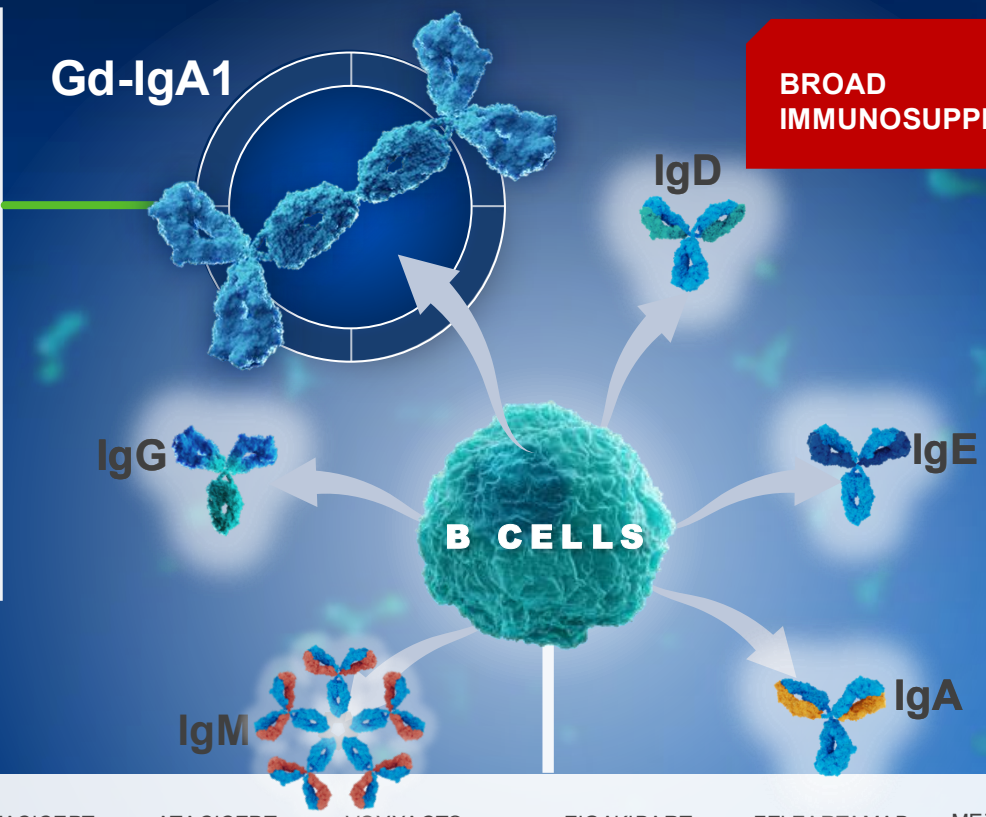



**Figure 3 | Treatment targets in immunoglobulin A nephropathy (IgAN) and the positioning of drugs included in this guideline.** Reflecting current understanding, Nefecon is shown as having a predominant effect on the production of pathogenic forms of IgA and IgA-containing immune complexes (IgA-ICs), with an undetermined direct effect of systemically absorbed budesonide on the kidneys. Systemic glucocorticoids have a well-documented anti-inflammatory effect within the kidneys and an undetermined direct effect on the production of pathogenic forms of IgA and IgA-ICs. Strategies to manage the generic response to IgAN-induced nephron loss may also include the use of 3-hydroxy-3-methyl-glutaryl-coenzyme A reductase inhibitors in selected patients. DEARA, dual endothelin angiotensin receptor antagonist; RASi, renin-angiotensin system inhibitor; SGLT2i, sodium-glucose cotransporter-2 inhibitor.

# BHV-1400: Selective Removal of Disease-Causing Gd-IgA1 Without Immunosuppression Compared to Market Competitors

DEGRADERS

TRAP™ Degrader
**BHV-1400 SELECTIVELY DEGRADES ONLY Gd-IgA1**
Targeting the pathogenesis of disease without immunosuppression




**BROAD IMMUNOSUPPRESSION**
TARPEVO®

**INHIBIT COMPLEMENT SYSTEM**
WITH BROAD IMMUNOSUPPRESSION
FABHALTA® · SEFAXERSEN · ULTOMIRIS®

**TARGET B CELLS**
WITH GLOBAL IMMUNOGLOBULIN SUPPRESSION
POVETACICEPT · ATACICEPT · VOYXACT® · ZIGAKIBART · FELZARTAMAB · MEZAGITAMAB

**TARGET ENDOTHELIN RECEPTOR**
FILSPARI® · VANRAFIA®

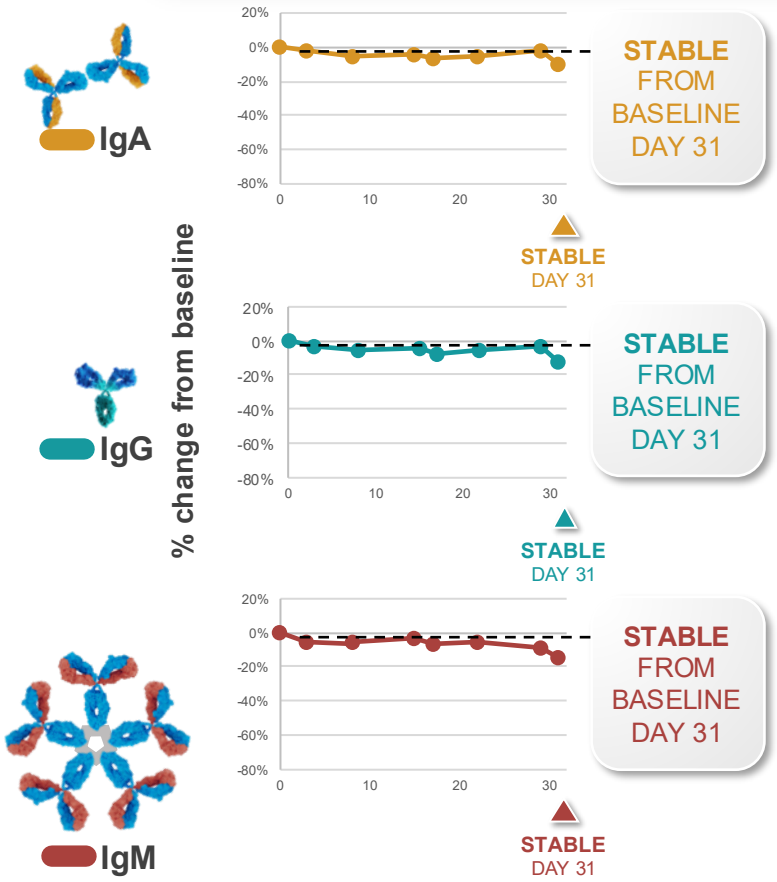
**KEY POINT**

BHV-1400 is the only therapy designed to remove pathogenic Gd-IgA1, the root cause of IgAN, while preserving healthy immune function

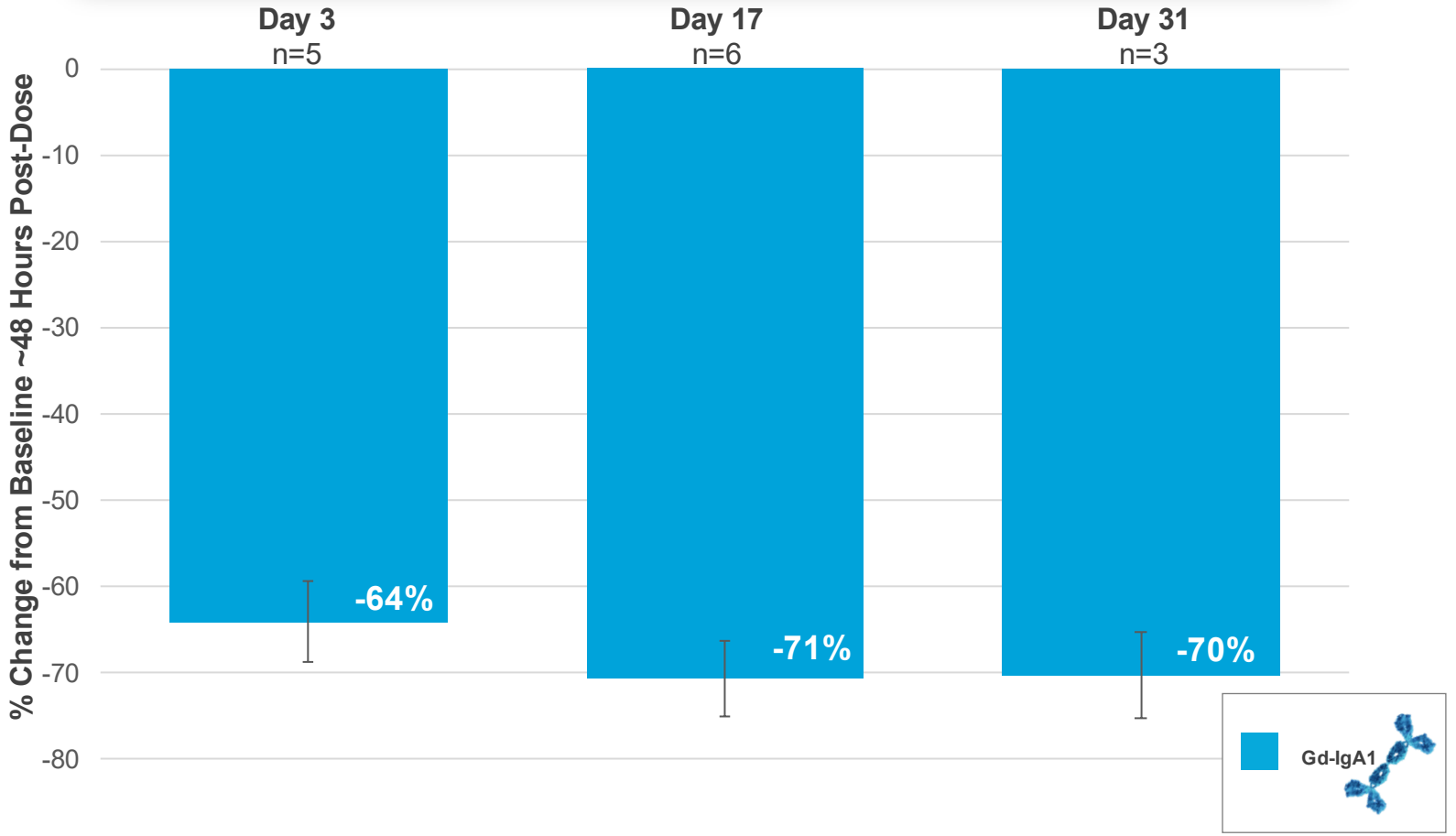
# BHV-1400 500 mg SC Bi-Monthly Deeply and Selectively Removes Gd-IgA1 Without Suppression of Normal Healthy IgA in Patients With IgAN

DEGRADERS

## Healthy Immunoglobulins




## Gd-IgA1




Preliminary data from ongoing study. Data represents mean % change in immunoglobulins in patients with IgAN administered one month of BHV-1400 500 mg every two weeks. Error bars represent standard error.

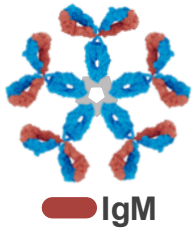
# Competitors Show Long-Term Immunosuppression[1,2]

DEGRADERS

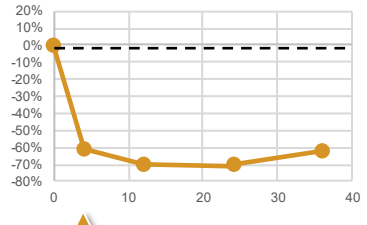






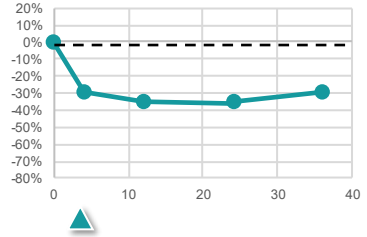



## Mezagitamab 600 mg SC[2,4]




**61% DROP**
MONTH 4

**62% DROP** FROM BASELINE MONTH 36

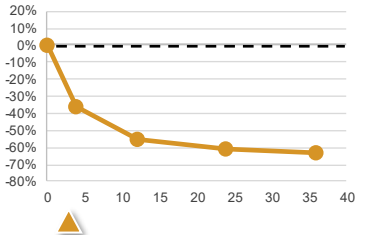



**30% DROP**
MONTH 4

**30% DROP** FROM BASELINE MONTH 36

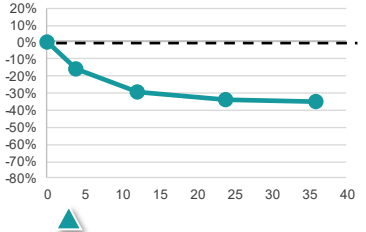
IgM Data not Reported

## Atacicept 150 mg[2,5]



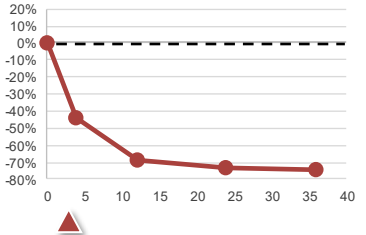

**36% DROP**
DAY 30

**64% DROP** FROM BASELINE WEEK 36




**16% DROP**
DAY 30

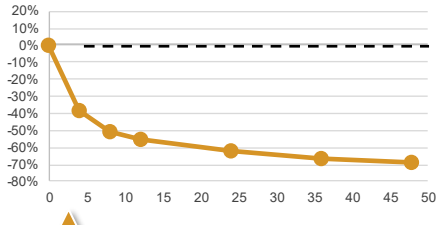
**35% DROP** FROM BASELINE WEEK 36




**44% DROP**
DAY 30

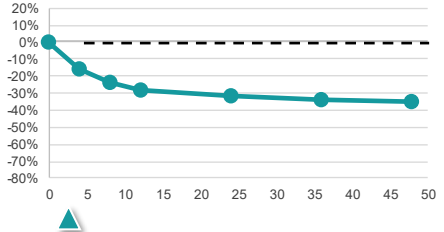
**75% DROP** FROM BASELINE WEEK 36

## VOYXACT SC[2,6]



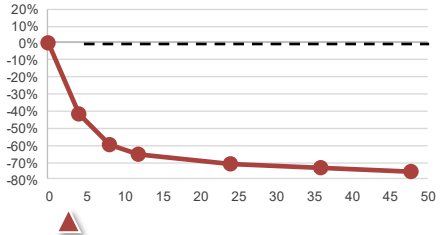

**38% DROP**
DAY 30

**69% DROP** FROM BASELINE WEEK 48




**16% DROP**
DAY 30

**35% DROP** FROM BASELINE WEEK 48




**42% DROP**
DAY 30

**75% DROP** FROM BASELINE WEEK 48

VOYXACT FDA LABEL[7]

### WARNINGS AND PRECAUTIONS

**Immunosuppression and Increased Risk of Infections**
VOYXACT suppresses the immune system by reducing antibody production, which may increase the risk of infections.

**Immunosuppression and Immunization Risks**
Because of its mechanism of action, VOYXACT may interfere with the immune responses to vaccines and increase the risk of infection from live vaccines.

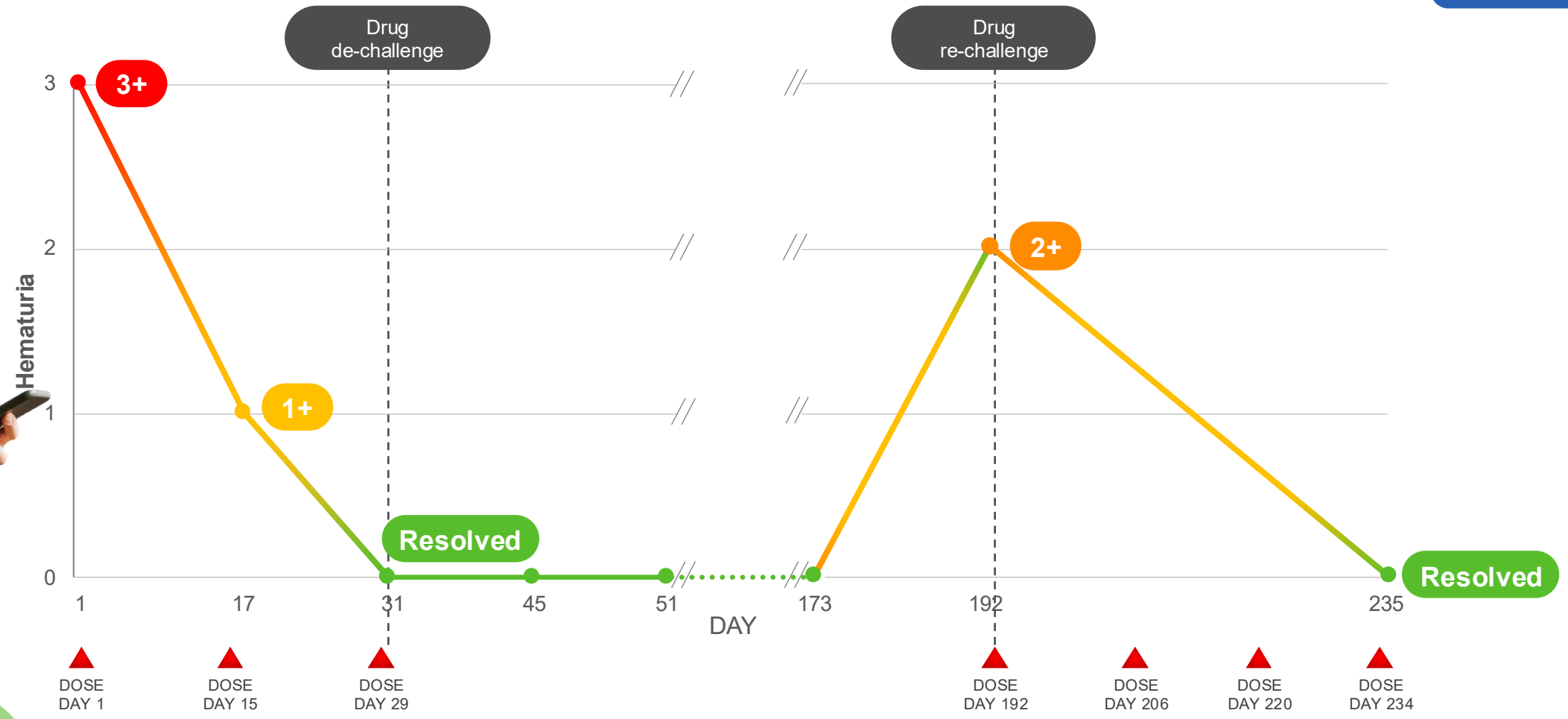
**Months** **Weeks** **Weeks**

**1.** Competitors did not report IgE. **2.** All competitor data presented herein are derived from publicly available sources only. Certain data points have been reconstructed or estimated from published graphical information. No confidential, non-public, or proprietary information was used. This analysis has not been reviewed or validated by the referenced companies. **3.** Solid dots represent the mean of the maximal total IgG % change from baseline 4. Barratt. American Society of Nephrology Kidney Week 2025. Poster FP-PO0808. **5.** Lafayette. Kidney International. 2024 **6**. Lafayette. NEJM. 2025. **7**. https://www.accessdata.fda.gov/drugsatfda_docs/label/2025/761434s000lbl.pdf

# BHV-1400 Hematuria Case Report: De-Challenge Re-Challenge Recaptures Complete Hematuria Resolution

DEGRADERS



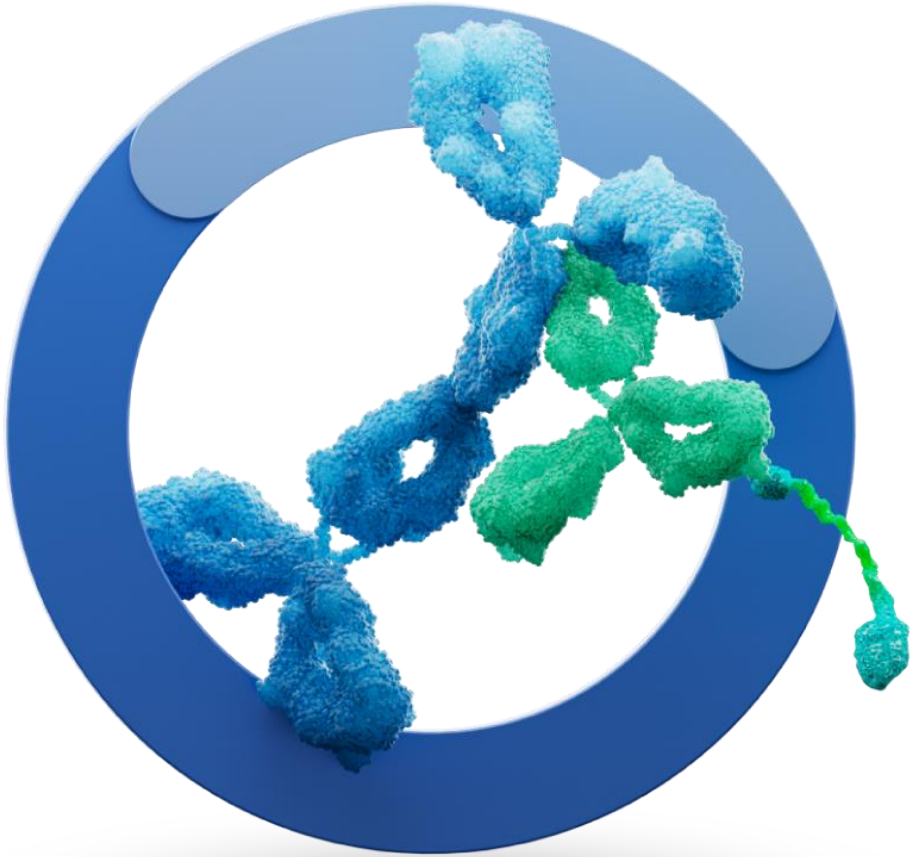



**KEY POINTS**

- Re-treatment with BHV-1400 500 mg every two weeks initiated upon disease recurrence after extended off-treatment period
- Complete resolution of hematuria re-achieved within weeks of reinitiation

# BHV-1400 Phase 1b IgA Nephropathy Patient Cohort — Safety Summary

DEGRADERS



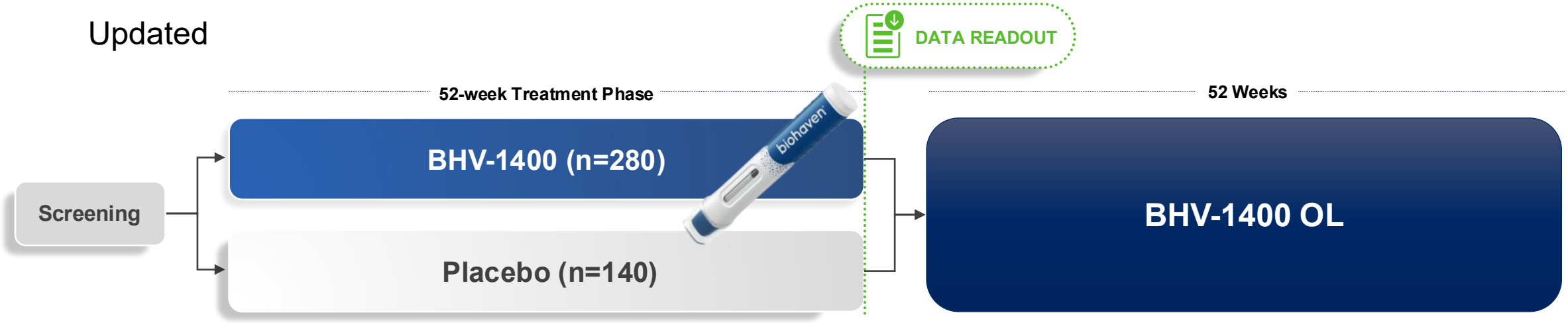
- 10 participants with IgA nephropathy
- Most AEs were mild and self-resolving
- No treatment discontinuations for adverse events
- No clinically significant trends in vitals, ECGs, or labs (including AST/ALT/Tbili)
- No clinical evidence of cardiovascular, renal, hepatic, or hematologic toxicity
- Preservation of IgA, IgG, IgM, IgE
- No SAEs, severe AEs, or AEs resulting in discontinuation of therapy

Preliminary data from ongoing study, analysis conducted May 22, 2026.

# Lead TRAP Degrader BHV-1400 Planned Phase 3 Initiation in 2026



Updated




## KEY STUDY DETAILS

**Study Design:** Randomized, double-blind, placebo-controlled trial

**Population:** Male and female adults with biopsy-proven IgAN

**Dose:** 500 mg self-administered via autoinjector

**Endpoints:** Δ UPCR, Δ in eGFR, Δ in Gd-IgA1 at week 52

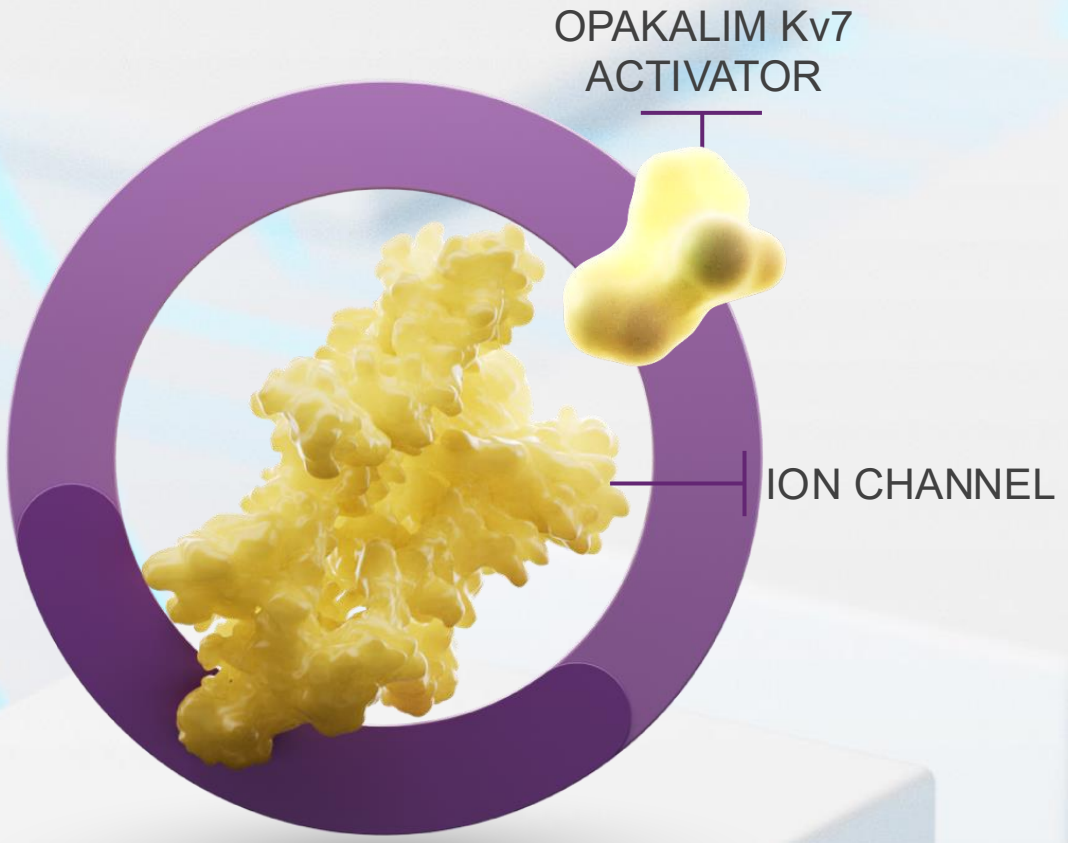
**KEY POINT**

BHV-1400 pivotal trial in IgA nephropathy initiating in 2026

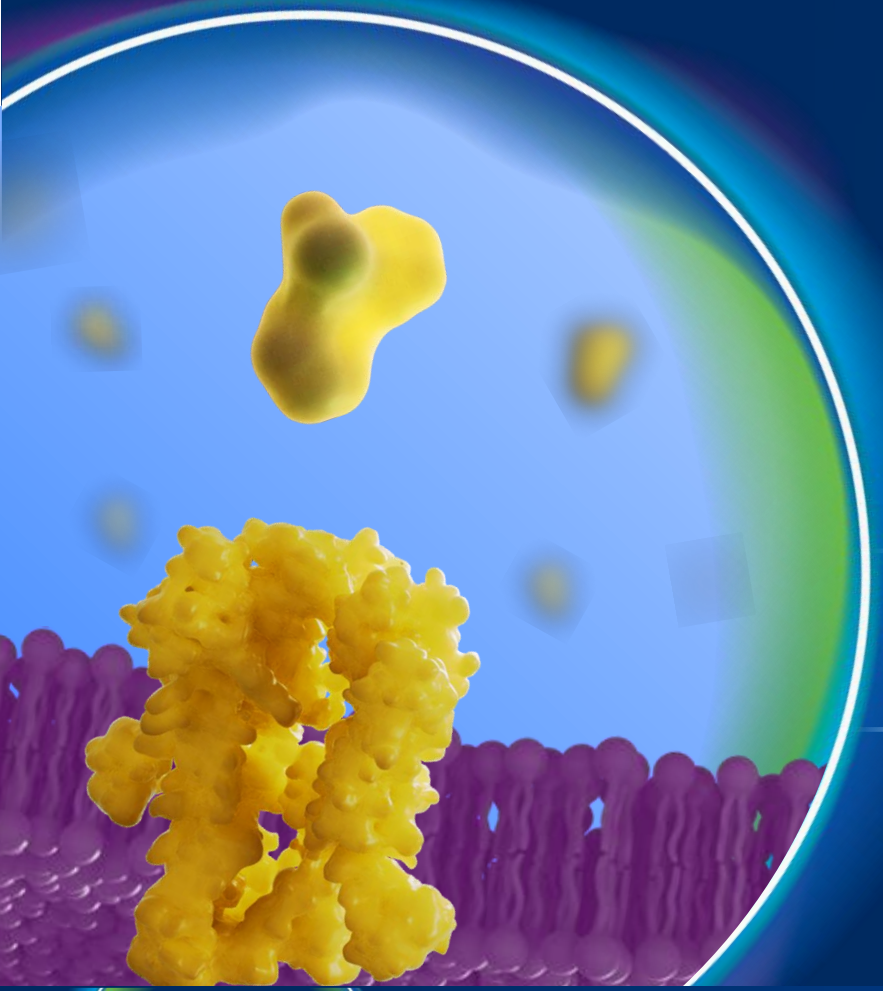
biohaven®

**SELECTIVE Kv7 ACTIVATOR:**
**OPAKALIM**

# Revolutionizing Epilepsy Treatment With a Modern Kv7 Activator



# An Epilepsy Treatment Designed With Patients and Physicians in Mind



**Opakalim offers potential for easy-to-use, once-daily treatment with no titration to control seizures without the burdensome side effects frequently reported with approved ASMs and those in development**

**Selectively activates Kv7.2/7.3 channels—a validated MOA for treating epilepsy—without impacting GABA receptors**

**Exhibits preliminary efficacy signals in focal epilepsy OLE, KCNQ2-DEE and now idiopathic generalized epilepsy**

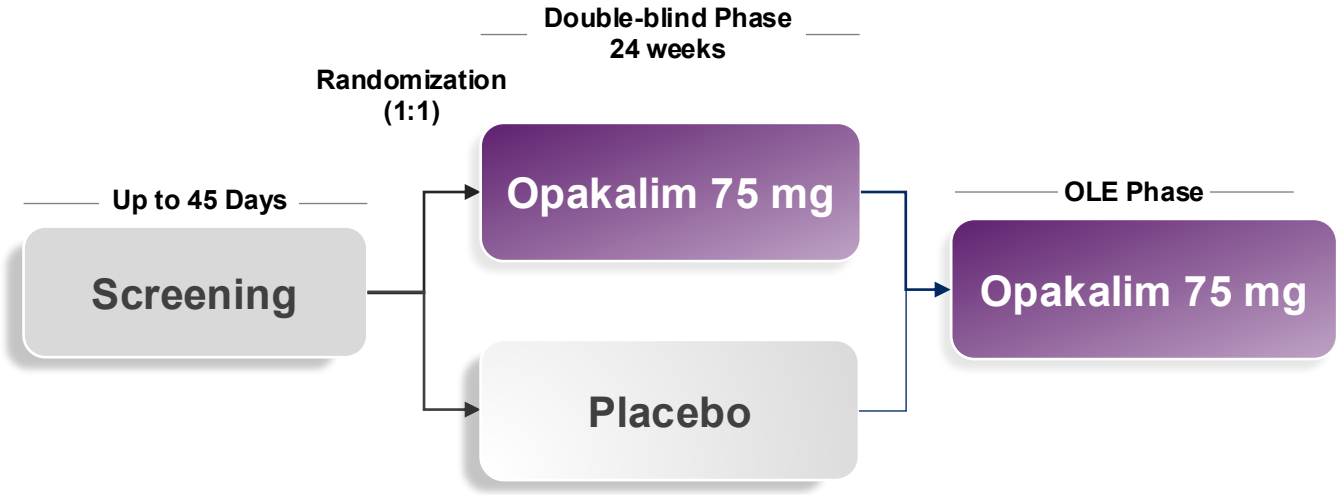
**Demonstrates exceptional safety profile with low rates of CNS adverse events across all trials (1300+ subjects)**

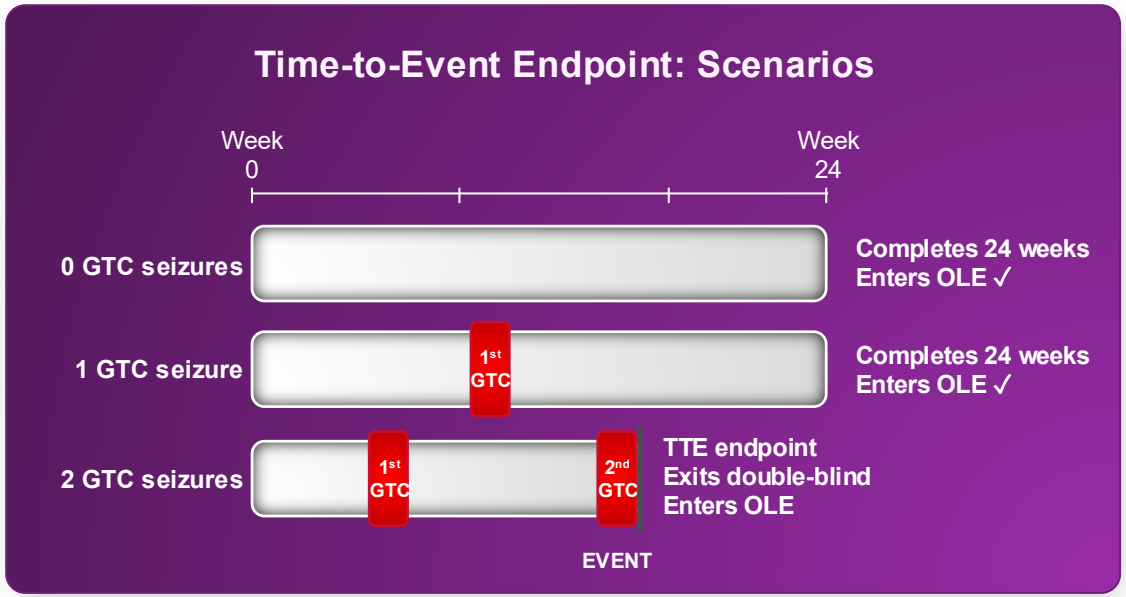
BREAKING **NEWS**

- Announced exclusive global license with SK Biopharmaceuticals
- 1st focal epilepsy study topline expected 2H2026

# Idiopathic Generalized Epilepsy Time-to-Event Study Design

Kv7




## KEY STUDY DETAILS

**Study Design:** Randomized, double-blind, placebo-controlled, event-driven trial

**Endpoint: Primary -** Time-to-event (Event = 2nd day with GTC seizure), **Secondary -** GTC seizure freedom

**Population:** Subjects 18–75 with IGE and intractable GTC seizures

**Key Entry Criteria:** 3 GTC seizures within the historic 16-week seizure assessment period

Study terminated early due to enrollment and strategic portfolio prioritization; GTC: generalized tonic-clonic; IGE: idiopathic generalized epilepsy; TTE: time-to-event

# Demographics and Baseline Disease Characteristics

Kv7

| | Opakalim 75 mg n=15 | Placebo n=12* |
|---|---|---|
| **Age (mean)** | **37** | 43 |
| **Sex (% female)** | **73%** | 83% |
| **Region (% US)** | **47%** | 25% |
| **BMI (mean)** | **27** | 28 |
| **Number of epilepsy treatments at screening** | | |
| **1 to 2** | **60%** | 67% |
| **3 to 4** | **40%** | 33% |
| **Number of previous and current ASMs** | | |
| **≤ 6** | **11 (73%)** | 10 (83%) |
| **> 6** | **4 (27%)** | 2 (17%) |
| **Age at IGE diagnosis (mean)** | **14** | 13 |
| **Years since IGE diagnosis (mean)** | **23** | 30 |

* In placebo group, 1 subject did not have post-dose efficacy data

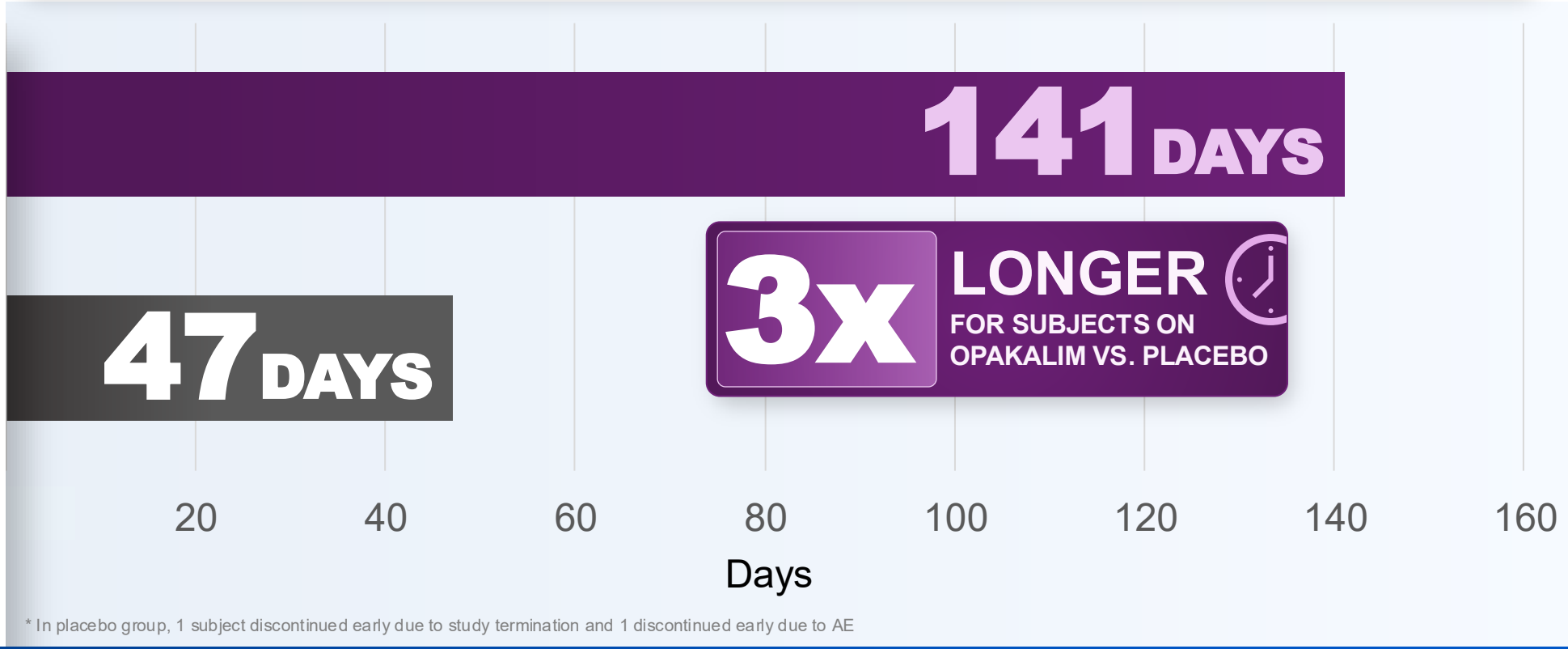
KEY POINT

Highly treatment-resistant idiopathic generalized epilepsy population

# Opakalim Prolongs Time-to-2nd GTC Seizure

Kv7

MEDIAN TIME-TO-2ND GTC SEIZURE



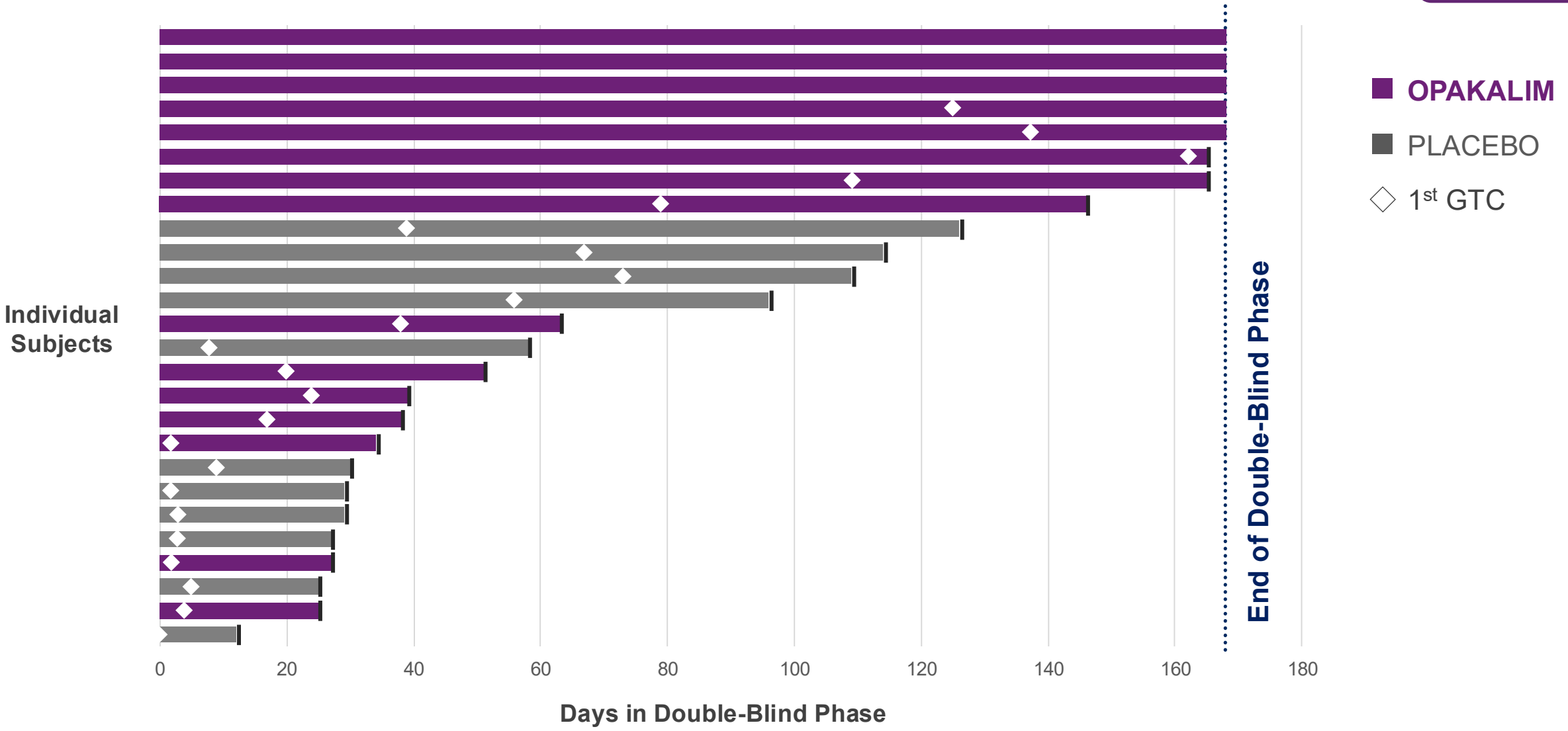

* In placebo group, 1 subject discontinued early due to study termination and 1 discontinued early due to AE

KEY POINT

Efficacy signal observed in idiopathic generalized epilepsy population

# Opakalim Prolongs Time-to-2nd GTC Seizure and Time in Double-Blind

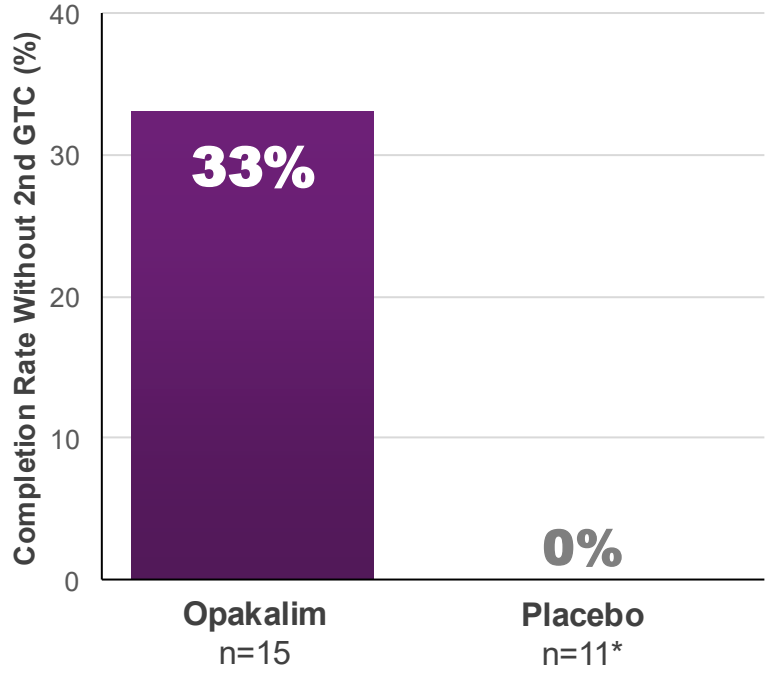
Kv7



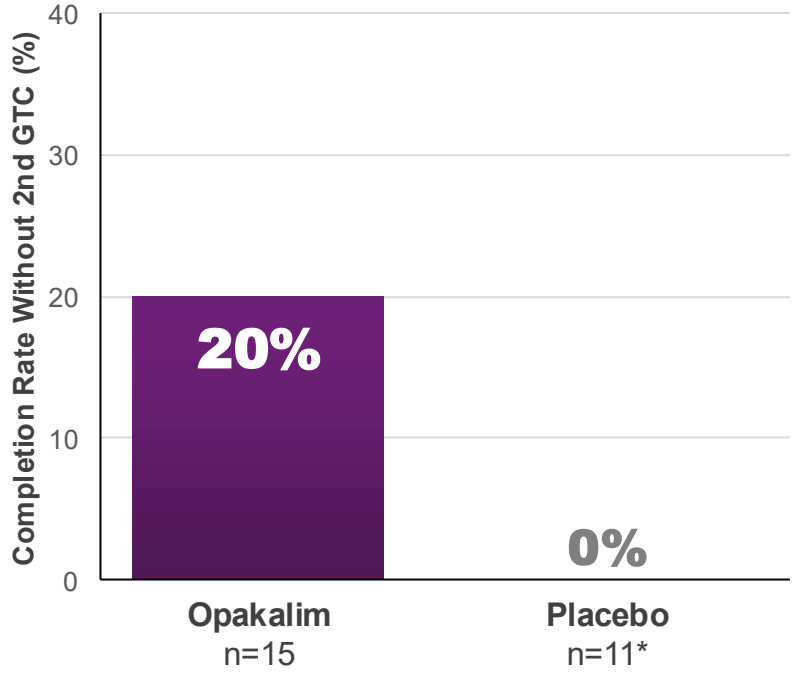
# Opakalim Prolongs Time in Double-Blind and Enhances Seizure Control

Kv7

## 33% of Opakalim-Treated Subjects Completed Six-Month Double-Blind Phase Without 2nd GTC




## 20% of Opakalim-Treated Subjects Completed Double-Blind Phase Seizure Free

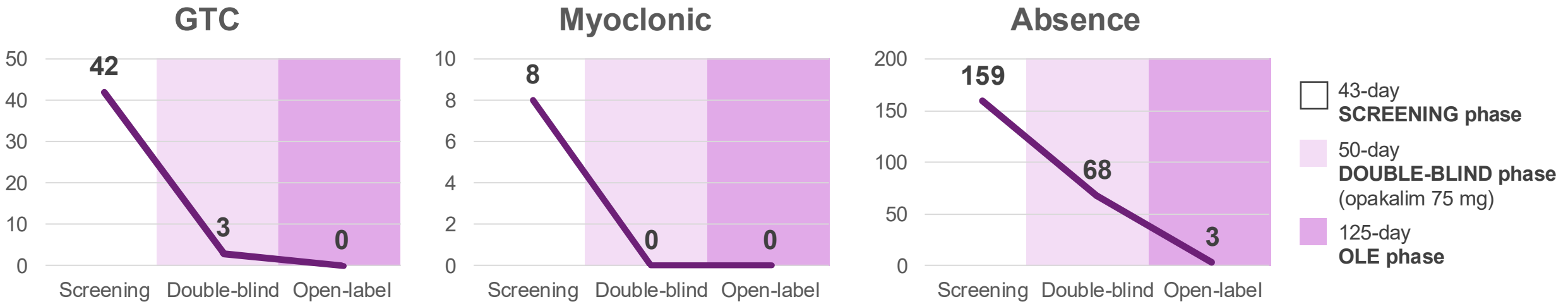



* In placebo group, 1 subject discontinued early due to study termination and 1 discontinued early due to AE

# Promising Case of Broad-Spectrum Generalized Seizure Efficacy

Kv7

*Our patient has very difficult-to-treat IGE, since entering the opakalim trial, she has experienced reduction in all seizure types.*




*In terms of tolerability, she continues to do very well, with no adverse events.*

Opakalim IGE Double-Blind Phase topline data, 1H 2026

**KEY POINTS**

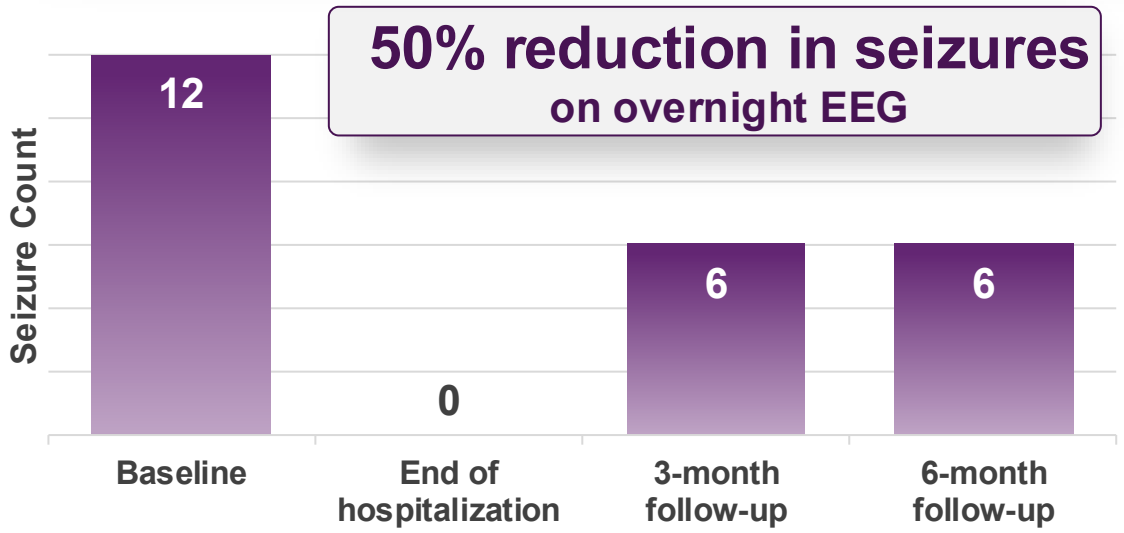
- 50% of subjects with myoclonic seizures became myoclonic seizure free on opakalim
- 33% of subjects with absence seizures became absence seizure free on opakalim

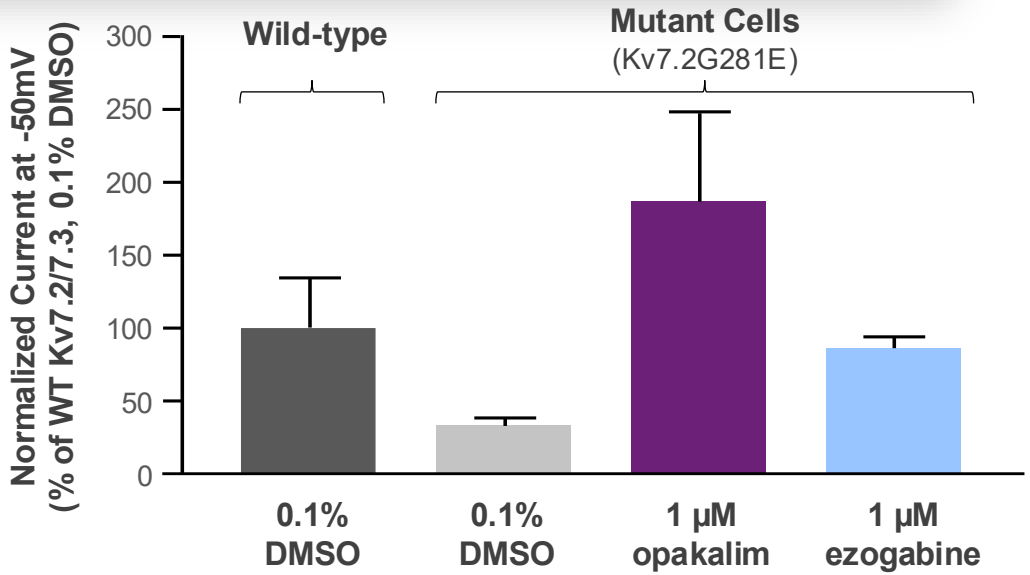
# Efficacy Signal Demonstrated in KCNQ2-DEE



**9-year-old boy with**
- **Refractory KCNQ2-DEE**
- **Kv7 activation-dependence**

- Heterozygous for Kv7.2 G281E mutation
- Daily tonic seizures at baseline despite 3 ASMs including 1st gen Kv7 activator
- Prior attempts to taper 1st gen Kv7 activator resulted in **status epilepticus, ICU admission and developmental regression**




Olson. AAN 2026. Poster #P10 11-002; Equivalent exposures to 75 mg dose in pivotal focal epilepsy studies

**KEY POINT**

Successfully transitioned from 1st gen Kv7 to opakalim and stable for 6+ months

# Efficacy Signal Observed in Focal Epilepsy Open-Label Data

Kv7




**SEIZURE FREQUENCY**
**Pretreatment Baseline in OP**

vs.

**SEIZURE FREQUENCY**
**On Treatment with Opakalim 75 mg in OLE**

**>54%**
OF PATIENTS SHOWED

**50%**
**RESPONSE RATE**

OVER ANY CONSECUTIVE 6-MONTH PERIOD IN OLE (n>100)

RISE 2 Part B: opakalim 75 mg;; Opakalim ongoing focal epilepsy preliminary data 1H 2026 for open-label 6-month completers; French. Epilepsia Open. 2025; Indirect comparisons between compounds based on publicly available data.
As part of the nonclinical characterization of certain metabolites of opakalim observed in rodent testing, an update was provided to global regulatory authorities regarding these ongoing studies. It was determined that there was insufficient information related to a specific metabolite to assess its risk to human subjects. The FDA instructed Biohaven to pause new enrollment until additional clarifying nonclinical studies are completed and issued a partial clinical hold letter on September 4, 2026.

**KEY POINT**

50% RR for opakalim (54%) comparable to azetukalner (56%)

# Exceptional Tolerability Observed in Focal Epilepsy Open-Label Data

Kv7

| Preferred Term | Opakalim 50 mg | Opakalim 75 mg | Opakalim Pooled |
|---|---|---|---|
| Headache | 4.5% | 6.4% | 5.7% |
| Nasopharyngitis | 4.5% | 6.4% | 5.7% |
| Seizure | 5.3% | 3.7% | 4.3% |
| Dizziness | 3.0% | 5.0% | 4.3% |
| Fatigue | 3.0% | 4.1% | 3.7% |
| Fall | 2.3% | 4.6% | 3.7% |
| Upper respiratory tract infection | 3.0% | 4.1% | 3.7% |
| Back Pain | 3.8% | 3.2% | 3.4% |
| Insomnia | 5.3% | 2.3% | 3.4% |
| Nausea | 3.8% | 2.8% | 3.1% |
| Diarrhea | 6.0% | 1.4% | 3.1% |

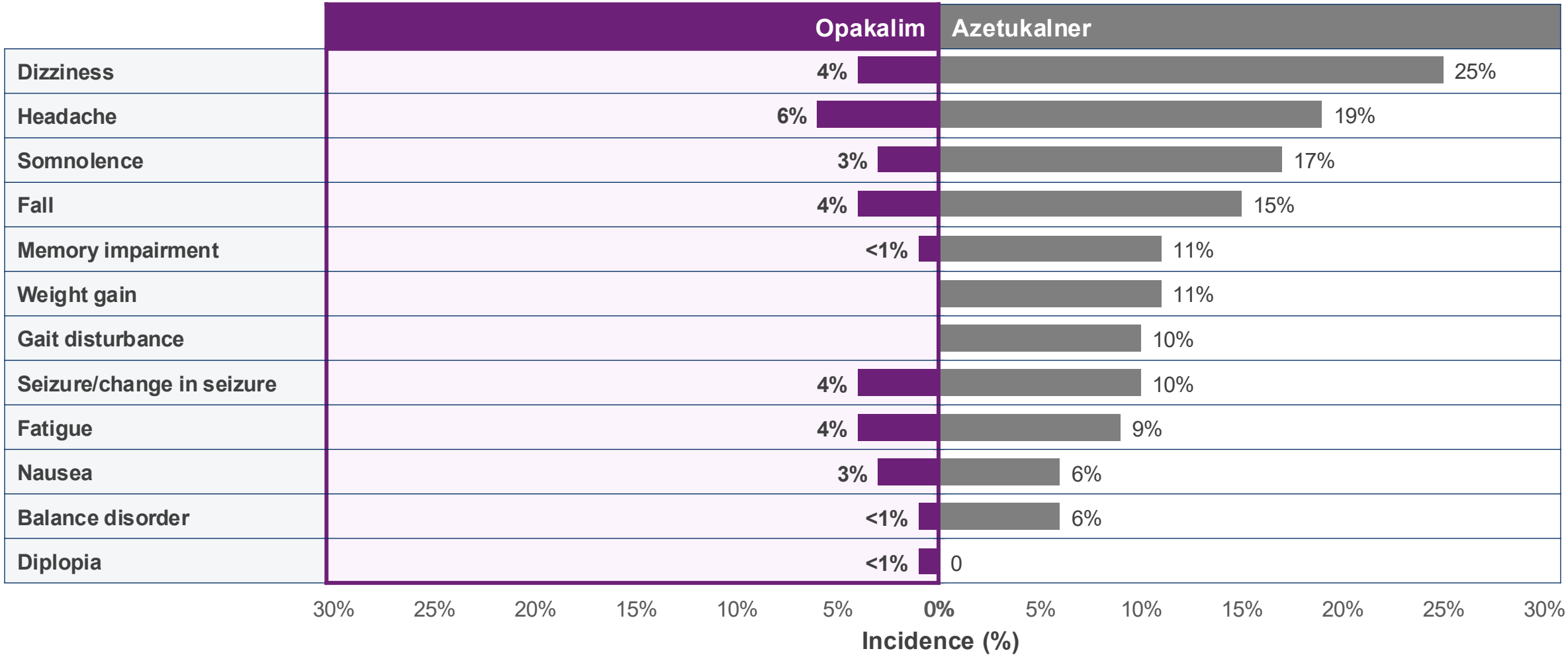
Adverse events reported in ≥3% of pooled participants, opakalim ongoing focal epilepsy open-label preliminary data 1H 2026

KEY POINT

Low incidence, majority mild and spontaneously resolved

# Opakalim Demonstrates Favorable Tolerability vs. Azetukalner in Focal Epilepsy Open-Label Trials

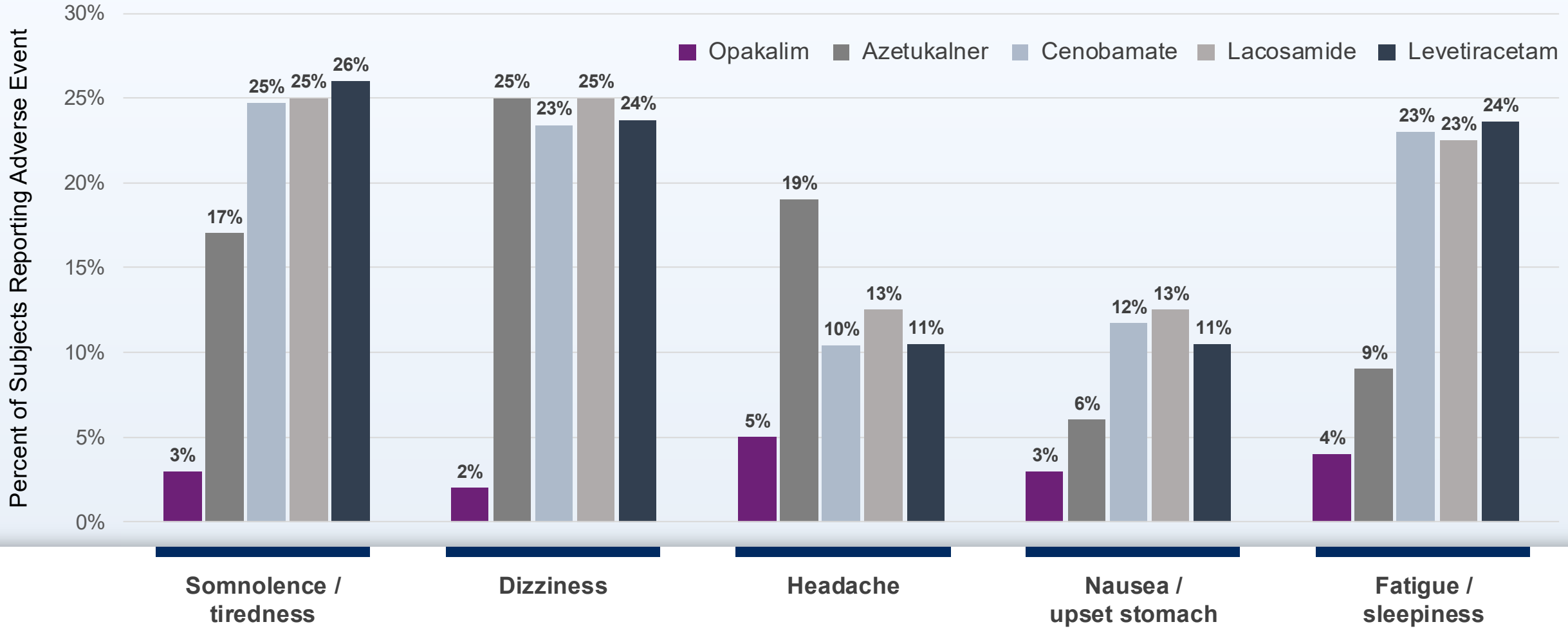
Kv7




Opakalim ongoing focal epilepsy open-label preliminary data 1H 2026; percentages rounded to the nearest whole percent; Azetukalner focal epilepsy data from Open Label Study – French. AES 2025. Poster #3.356.

# Opakalim Demonstrates Favorable Tolerability vs. Approved and Investigational ASMs

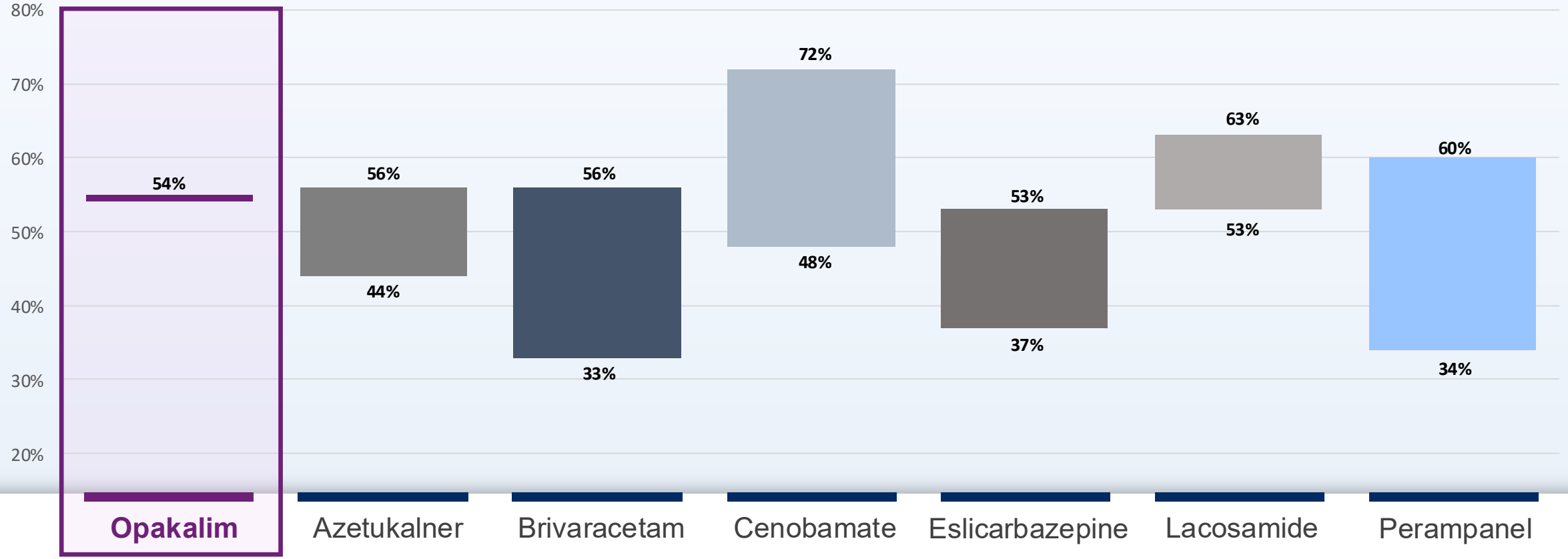
Kv7




Opakalim ongoing focal epilepsy open-label preliminary data 1H 2026; Azetukalner data from Open Label Study – French. AES 2025. Poster #3.356; Cenobamate, lacosamide and levetiracetam data from Winter. CNS Drugs. 2024.

# 50% Responder Rate in OLE Trials of Several Approved and Investigational ASMs

Kv7




Opakalim ongoing focal epilepsy open-label preliminary data 1H 2026; Hufnagel. Epilepsy Res. 2013; Halász. Epilepsia. 2010; Strzelczyk. Epilepsia. 2021; Ben-Menachem. Epilepsy Res. 2021; O'Brien. Epilepsia. 2020; Klein. Neurology. 2022; Strzelczyk. Expert Rev Clin Pharmacol. 2015; Husain. Epilepsia. 2012; French, J. Epilepsia Open. 2025; Rektor. Epilepsia. 2020.

**KEY POINT**

Opakalim OLE preliminary efficacy outcomes fall within reported range of responder rates for other ASMs in OLE trials

# Opakalim Is Easy-to-Use With a Projected Favorable Tolerability Compared to Approved ASMs

Kv7

| | | No titration | Favorable CNS tolerability | Low neuropsychiatric AEs | Low metabolic / electrolyte AEs | Low SJS or DRESS risk |
|---|---|---|---|---|---|---|
| L1 | Lamotrigine | ✗✗ | ✓ | ✓ | ✓ | ✗✗ |
| | Levetiracetam | ✓ | ✓ | ✗ | ✓ | ~ |
| | Oxcarbazepine | ✗ | ✗ | ✓ | ✗ | ✗ |
| L2 | Lacosamide | ✗ | ✓ | ✓ | ✓ | ✓ |
| | Eslicarbazepine | ✗ | ✗ | ✓ | ✗ | ~ |
| | Brivaracetam | ~ | ✗ | ✗ | ✓ | ✓ |
| L3 | Zonisamide | ✗ | ✗ | ✗ | ✗ | ✓ |
| | Cenobamate | ✗✗ | ✗ | ✓ | ✓ | ✗✗ |
| | Topiramate | ✗ | ✗ | ✗ | ✗ | ✓ |
| | **Opakalim (Kv7)** | ✓ | ✓ | ✓ | ✓ | ✓ |

✓ Favorable ~ Variable ✗ Unfavorable ✗✗ Very Unfavorable

**AE,** Adverse Event. **SJS,** Stevens-Johnson Syndrome. **DRESS,** Drug Reaction with Eosinophilia and Systemic Symptoms

# SK Biopharmaceuticals | Biohaven Global License for Opakalim

**30+ years of history**

**150+ professionals** in a dedicated US neurology field force

**Double-digit** steady growth of XCOPRI®

SK biopharmaceuticals

**THE TEAM THAT LAUNCHED XCOPRI®**



**PAYMENTS TO BIOHAVEN**

| | |
|---|---|
| **$400M** | Upfront: $350M at Closing, $50M one year after Closing |
| **Up to $150M** | Contingent development and regulatory milestones |
| **Mid-teens to low twenties** | Tiered US opakalim royalties; mid-single-digit outside the US |

- First Korean company to independently discover, develop and commercialize a novel antiseizure medicine
- XCOPRI® marketed in the US for partial-onset (focal) seizures in adults
- SKBP CEO: Opakalim is "a highly complementary addition to our epilepsy portfolio"

**HSR waiting period has cleared and the parties are coordinating on the closing. Both parties have agreed to close the transaction on December 3, 2026, in accordance with the terms and conditions of the agreement**

## Additional deal terms assumed by SKBP

- **Go-forward costs** SKBP to fund specified Kv7 program costs
- **Up to $245M** Milestones to Knopp assumed by SKBP; separate from SKBP's payments to Biohaven

Royalties are subject to specified reductions and adjustments. Source: Biohaven Form 8-K and Exhibit 99.1 (August 26, 2026). XCOPRI is a registered trademark of SKBP.

biohaven®

**Biohaven and SKBP: advancing selective Kv7 activation in epilepsy**

biohaven®

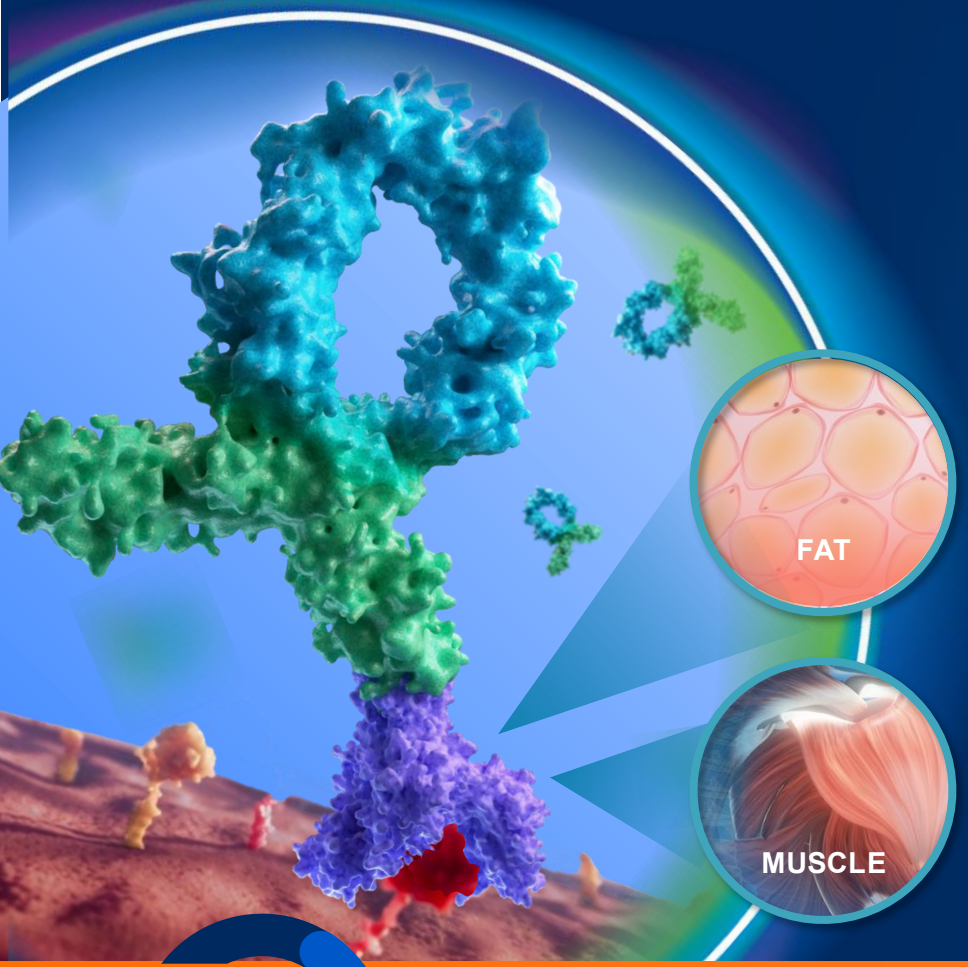
**MYOSTATIN ACTIVIN PATHWAY INHIBITOR:**
**TALDEFGROBEP ALFA**




# Targeting High-Quality Weight Loss in Obesity

# Targeting High-Quality Weight Loss With Myostatin-Activin Inhibition

TALDEFGROBEP




**Taldefgrobep directly targets fat and muscle while avoiding intolerable adverse effects**

**Novel myostatin-activin MOA for healthy weight loss**
Inhibits ActRII signaling in muscle and adipose tissue

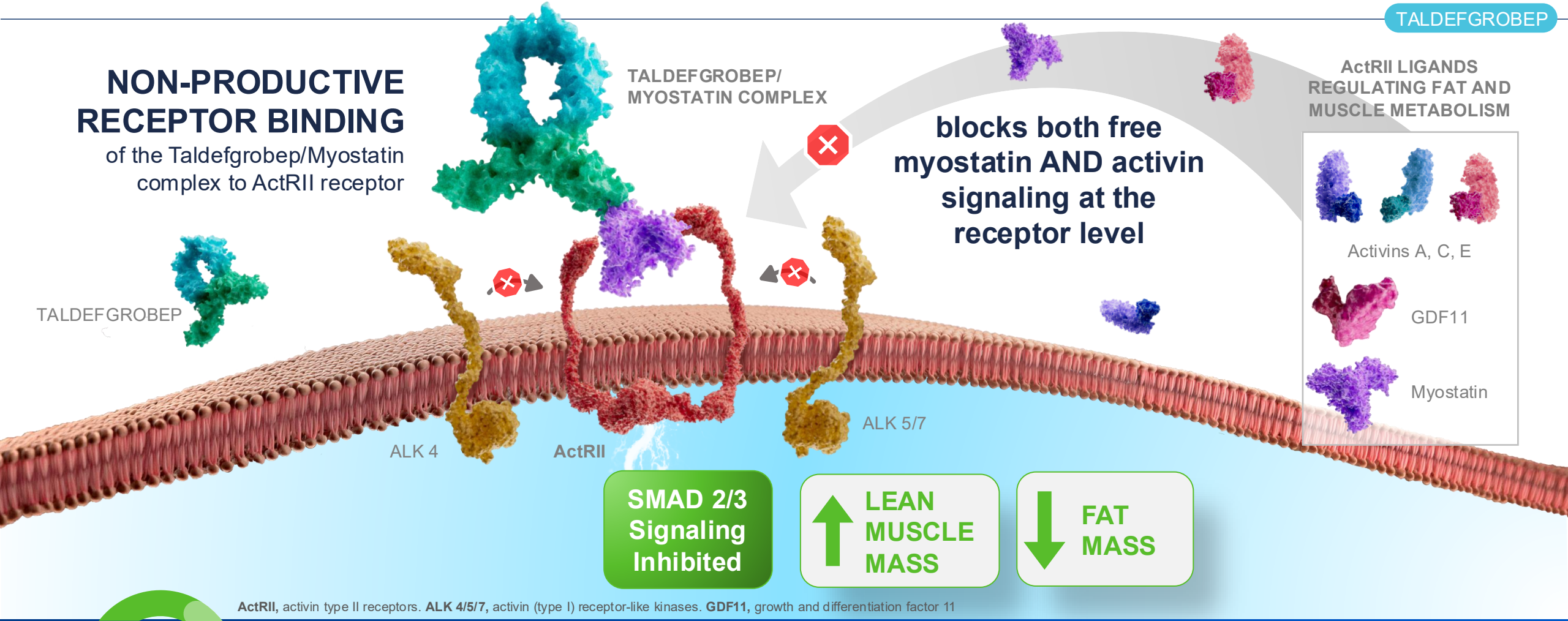
**Favorable safety profile established in >700 treated to date**
Low rates of muscle- and GI-related AEs

**Convenient dosing**
Administration by subcutaneous autoinjector

BREAKING NEWS

- Phase 2 study in obesity ongoing with planned addition of GLP-1 combination cohort
- Topline data from monotherapy and GLP-1 combination therapy arms expected 2H 2027

# Taldefgrobep Is a Novel Competitive Inhibitor of ActRII Signaling




**ActRII,** activin type II receptors. **ALK 4/5/7,** activin (type I) receptor-like kinases. **GDF11,** growth and differentiation factor 11

**KEY POINT**

Taldefgrobep binds ligands and competitively inhibits receptor activation in tissues where myostatin and multiple activins (e.g., A, C, E) are active

# Unmet Needs Remain in Obesity Management Despite New Therapies

TALDEFGROBEP

## Unprecedented reductions in total body weight are limited by:

### Significant muscle loss
Up to 40% of GLP-1-induced weight loss is lean mass[1,2]

### Increased Risk for Fractures
Pelvic fractures were 4–5x more common on Wegovy vs. PBO[3]

### Intolerable adverse events
Two-thirds stop GLP-1 therapy within 1 year[4]



## Opportunity in obesity management:

### Increase muscle mass and bone density while reducing fat mass
Including metabolically-active VAT

### Minimal or no clinically meaningful adverse events
Suitable for chronic administration

### Support convenient dosing
Potential for monthly dosing in an easy-to-use autoinjector for better adherence

**VAT**, Visceral Adipose Tissue.

**1.** Wilding. N Engl J Med. 2021. **2.** McCrimmon. Diabetologia. 2020. **3.** Wegovy USPI. Update NOV 2025. Accessed 29-DEC-2025. **4.** Prillman. Scientific American. 2024.

**KEY POINT**

Taldefgrobep targets the limitations of current therapies, offering a novel and differentiated solution for patients living with obesity

# Clinical Data Has Validated Myostatin Activin Inhibitor Class Uncovering Optimal Profile for Efficacy and Safety in Obesity

TALDEFGROBEP

| | Fat Reduction | Weight Loss | Favorable Safety Profile | Potential SC Monthly Dosing | MOA Directly Targeting: Adipose Tissue | MOA Directly Targeting: Skeletal Muscle |
|---|---|---|---|---|---|---|
| **Taldefgrobep** (Biohaven) | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ |
| **Bimagrumab** Activin Receptor Blocker (Lilly) | ✓ | ✓ | ✗ | ✗ | ✓ | ✓ |
| **Apitegromab*, SRK439** Anti-Myostatin (Scholar Rock) | ✗ | ✗ | ✓ | ✗ | ✗ | ✓ |
| **Trevogrumab*** Anti-Myostatin (Regeneron) | ✗ | ✗ | ✓ | ✗ | ✗ | ✓ |
| **Trevogrumab + Garetosmab*** Anti-Myostatin + Anti-Activin A (Regeneron) | ✓ | ✓ | ✗ | ✗ | ✗ | ✓ |
| **WVE-007, ARO-INHBE, ARO-ALK7** Activin E or ALK7 siRNA (Wave, Arrowhead) | ✓ | ? | ? | ✓ | ✓ | ✗ |

*Apitegromab, Trevogrumab and Trevogrumab + Garetosmab Phase 2 studies were conducted in combination with a GLP-1 agent.

KEY POINT

Taldefgrobep offers optimal mechanistic profile for efficacy and safety

# Myostatin Activin Pathway Inhibition Demonstrates Benefits in Total Body Weight, Fat and Muscle

TALDEFGROBEP

## BELIEVE: 1-Year Trial of Bimagrumab in Adults Living with Overweight or Obesity

| | Placebo | Bimagrumab (30 mg/kg iv) | Semaglutide (2.4 mg sc) | Combination (bima + sema) |
|---|---|---|---|---|
| **EFFICACY** | | | | |
| Total body weight (W48) | **-2.5%** | **-9.7%** | -14.3% | -20.2% |
| Total fat mass (W48) | -5.2% | **-25.3%** | **-24.8%** | -42.2% |
| Total lean mass (W72) | -0.5% | **2.5%** | -7.4% | -2.9% |
| Visceral Adipose tissue (W48) | -2.1% | **-40.2%** | -29.5% | -54.8% |

- Exceeds 5% differential guideline set by the FDA
- Change comparable to semaglutide
- Preservation/improvement in lean mass
- Greater reduction in metabolically-active VAT vs. semaglutide

**VAT**, Visceral Adipose Tissue.

KEY POINT

Taldefgrobep targets bimagrumab-like efficacy

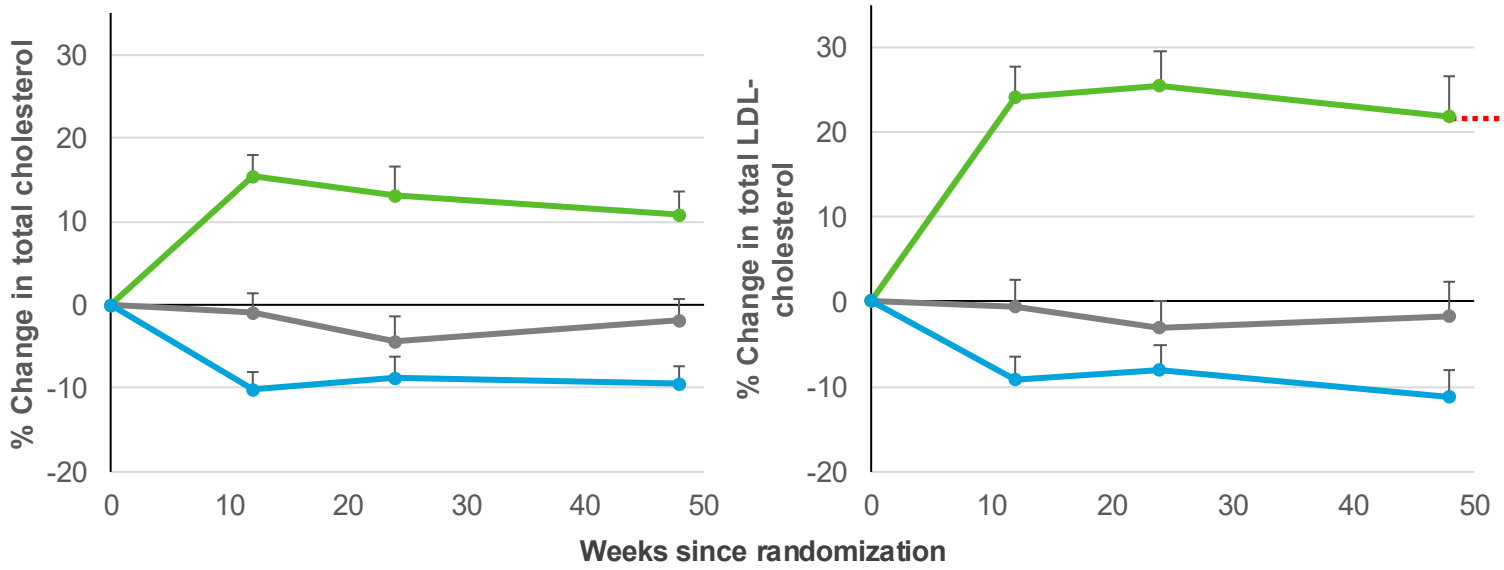
# Bimagrumab Provides Reason To "**Believe**" With Compelling Efficacy but Limited by Safety/Tolerability

TALDEFGROBEP

| | Placebo | Bimagrumab (30 mg/kg IV) | Semaglutide (2.4 mg SC) |
|---|---|---|---|
| Muscle Spasms | 5.5% | **73.7%** | 8.9% |
| Diarrhea | 5.5% | **49.1%** | 35.7% |
| Acne | 3.6% | **43.9%** | 8.9% |

**Poor tolerability due to irreversible ActRIIB binding**

Percent Change in Cholesterol From Baseline at 48 Weeks




**Elevation in lipids secondary to high peak exposures following IV administration**

**Bimagrumab** 30 mg/kg
**Semaglutide** 2.4 mg
Placebo

Heymsfield. Nature Medicine. 2026.

# Taldefgrobep Avoids GI- and Muscle-Related AEs Commonly Reported in Bimagrumab Clinical Trials

TALDEFGROBEP

| Muscle-/GI-Related AEs | **Taldefgrobep** SAD/MAD Pooled[1] 15-180 mg n=103 | **Bimagrumab** 30 mg/kg[2] Single Dose Study n=10 | **Bimagrumab** 10 mg/kg[3] Q4W Multi-dose Study n=37 |
|---|---|---|---|
| Acne | **0%** | **30%** | 3% |
| Muscle spasm | **3%** | **30%** | **41%** |
| Musculoskeletal stiffness | **0%** | **30%** | NA |
| Myalgia | **1%** | **30%** | NA |
| Muscle weakness | **1%** | **10%** | NA |
| Diarrhea | **2%** | **10%** | **41%** |
| Nausea | **1%** | NA | **11%** |
| Lipase level increased | **0%** | 0% | **11%** |

NA = data not available

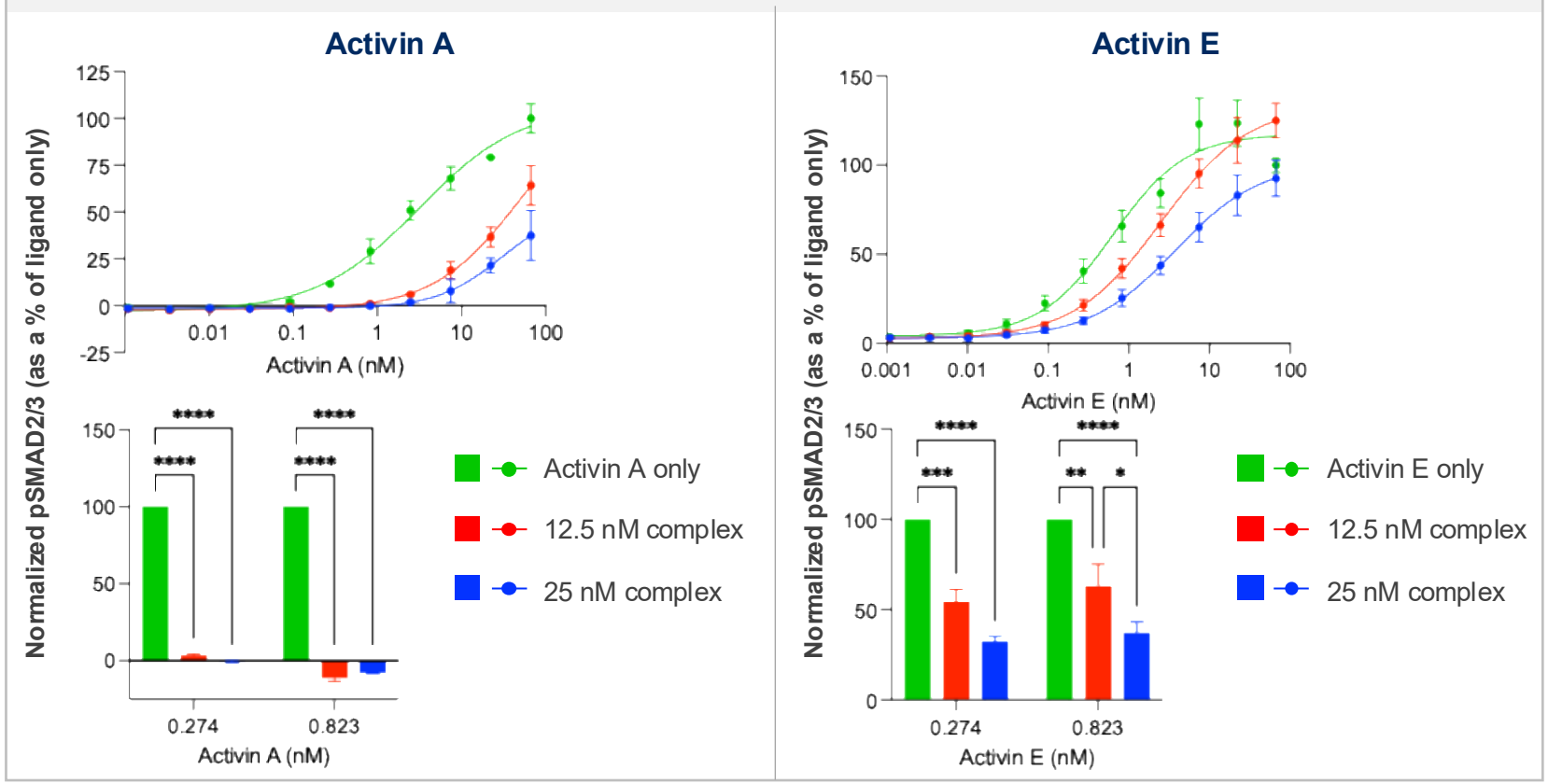
**1.** Study CN001001 conducted in healthy adults receiving taldefgrobep (15-180 mg QW x 1 month). **2.** Garito. Diabetes Obes Metab. 2018. **3.** Heymsfield. JAMA Network Open. 2021.

KEY POINT

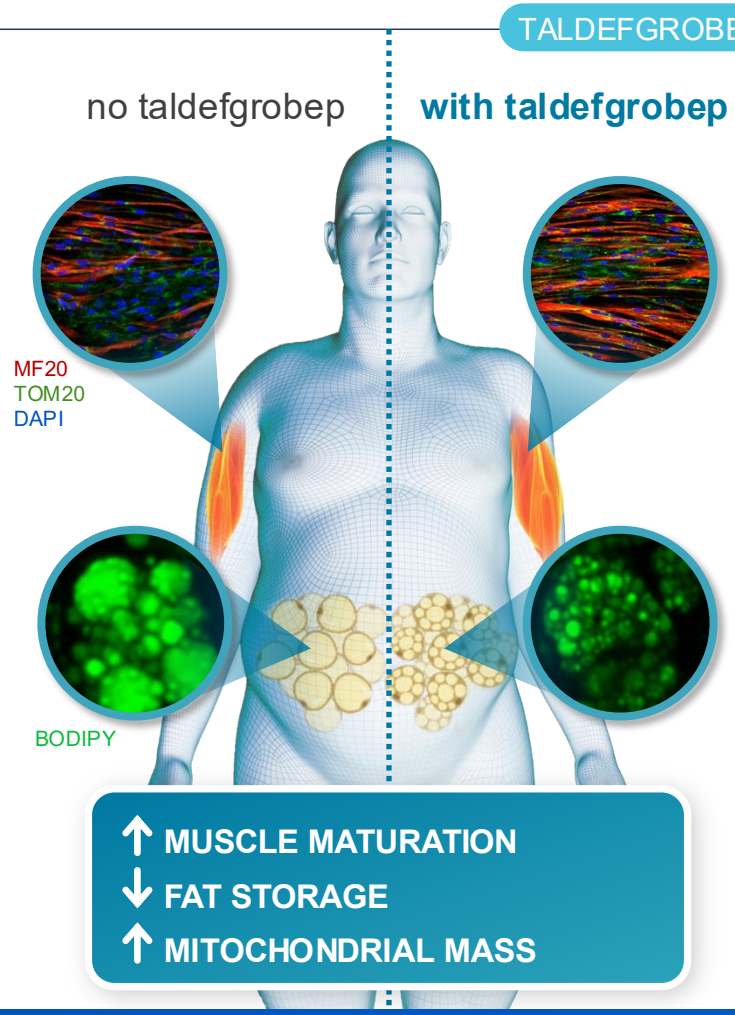
Favorable safety profile established in >700 participants across diverse clinical populations

# Taldefgrobep/Myostatin Complex Competes With Activin Signaling To Improve Muscle Differentiation and Fat Storage

## T/M Complex Inhibits Activin-Induced Signaling Through ActRII




*p<0.05, **p<0.01, ***p<0.001, ****p<0.0001



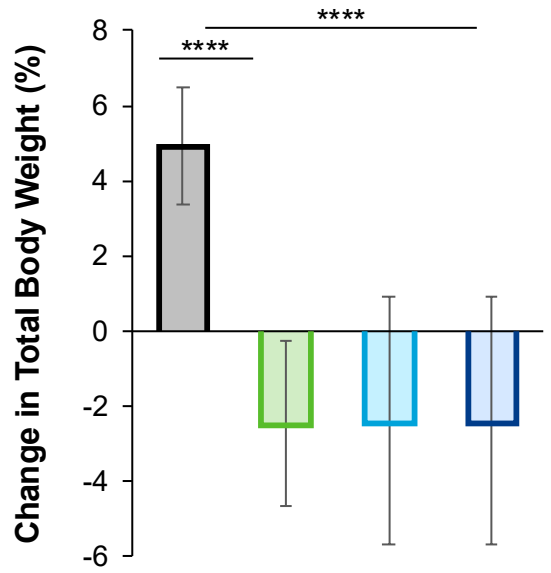

**KEY POINT**

The T/M complex, at clinically relevant exposures, inhibits activin A- and activin E-induced ActRII signaling

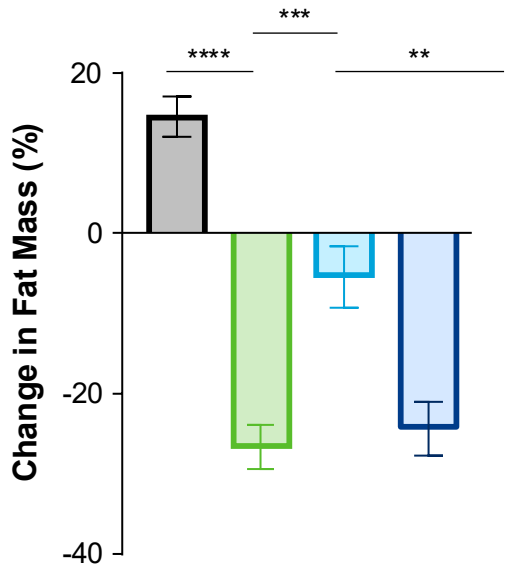
# In an Obese Mouse Model, Taldefgrobep Demonstrated Significant Reductions in Fat Mass While Increasing Lean Mass

TALDEFGROBEP

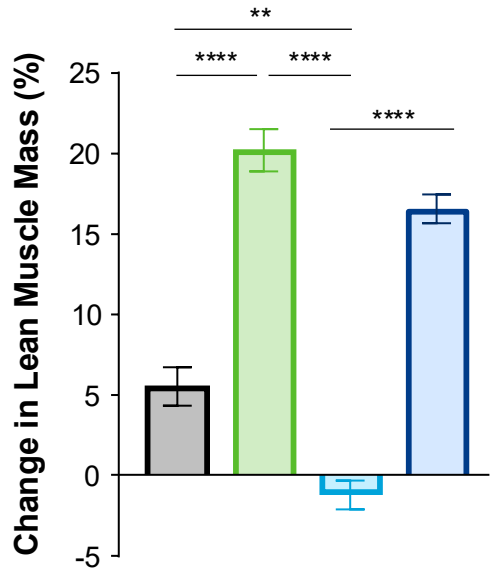
## Change in Total Body Weight, From Baseline at 8 Weeks




## Change in Fat Mass From Baseline at 8 Weeks




## Change in Lean Muscle Mass From Baseline at 8 Weeks




Vehicle | Taldefgrobep | Semaglutide (40 µg/kg QD) | Taldefgrobep + Semaglutide (40 µg/kg QD)

n=15 for vehicle; n=16 for all other groups. Error bars represent standard error of the mean. Significance evaluated using Tukey's multiple comparisons test. **P < 0.01; ***P < 0.001; ****P < 0.0001; **QD,** once daily; taldefgrobep alfa. Bechtold. ObesityWeek 2024. Poster 350.

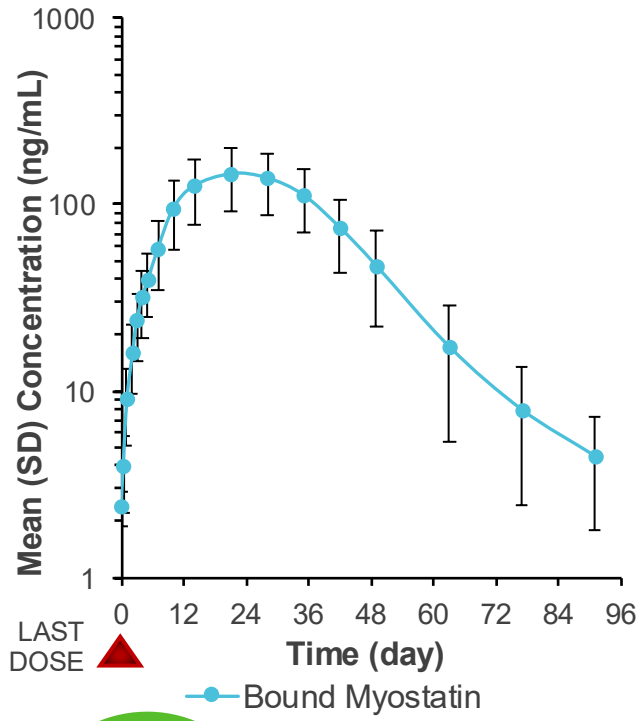
**KEY POINT**

Taldefgrobep demonstrated reductions in fat mass and increases in lean mass as monotherapy and in combination with a GLP-1 receptor agonist

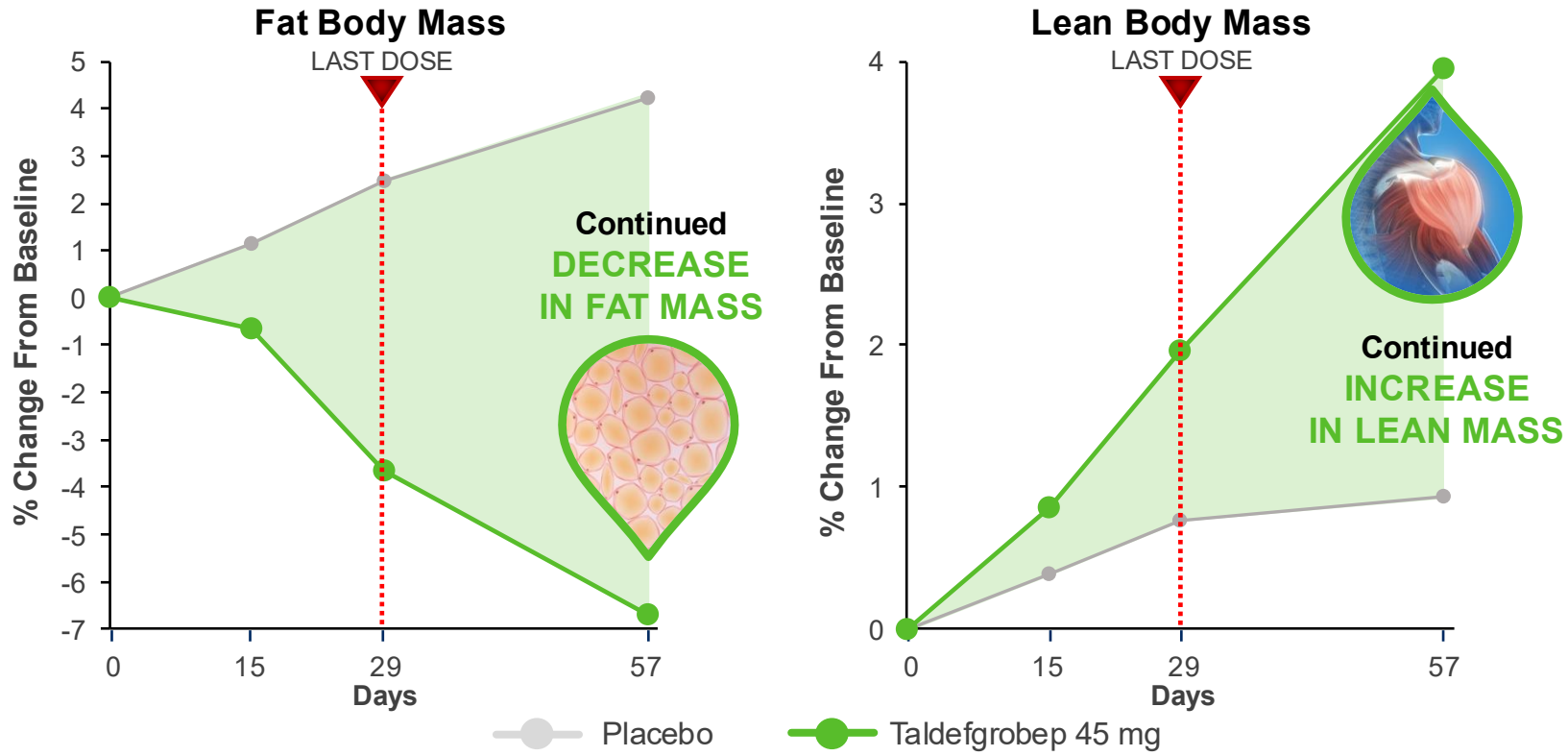
# Taldefgrobep Improves Body Composition in Non-Obese Adults With Potential for Monthly Dosing in Obesity

TALDEFGROBEP

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




In Healthy Adults at One Month Post Dosing, **Taldefgrobep** Demonstrates:




**KEY POINT**

Taldefgrobep-myostatin complex increases lean mass and also directly inhibits Activin E / ALK7 signaling in adipose tissue

# Taldefgrobep Has an Established, Favorable Safety Profile





Safety database includes **more than 700** treated trial participants



Assessed across a **wide dose range** (4 mg to 180 mg SC QW) and **broad demography**



Data from repeat dosing up to **192 consecutive weeks**

- **LOW RATES** of SAEs and AEs leading to discontinuation
- **LOW RATES** of GI- and muscle-related AEs commonly reported with other myostatin-activin pathway inhibitors
- **No identified serious signature clinical safety events**

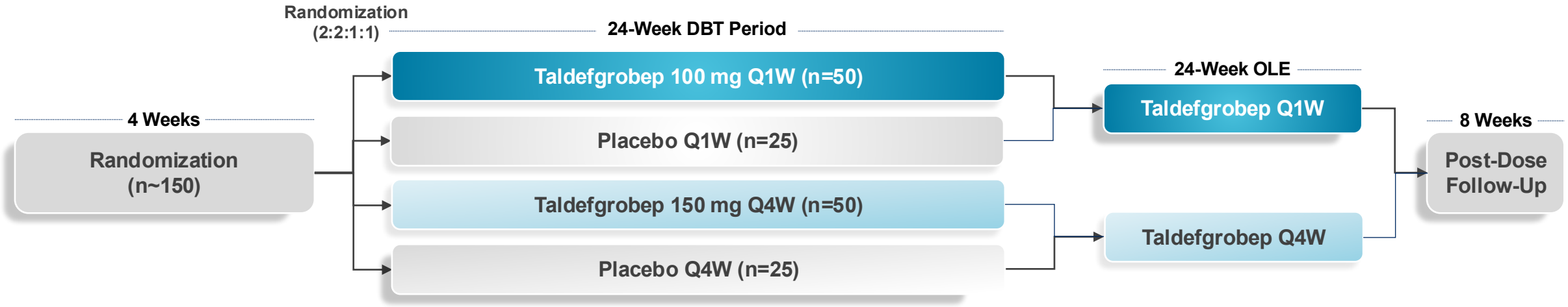
**KEY POINT**

Safety profile well-suited for indication in chronic weight management

# Ongoing Phase 2 Monotherapy Dose-Ranging (Q1W + Q4W) Study

TALDEFGROBEP

## PART A: Monotherapy




**KEY STUDY DETAILS**

**Study Design:** Phase 2, randomized, double-blind, placebo-controlled dose-ranging study

**Population:** Male and female adults (18 to 65 years old) with overweight or obesity

**Blinded Interim Analysis** (when 30% complete Week 12)**:** Taldefgrobep PK/PD (free myostatin and T/M complex concentrations) and plasma lipids by treatment assignment

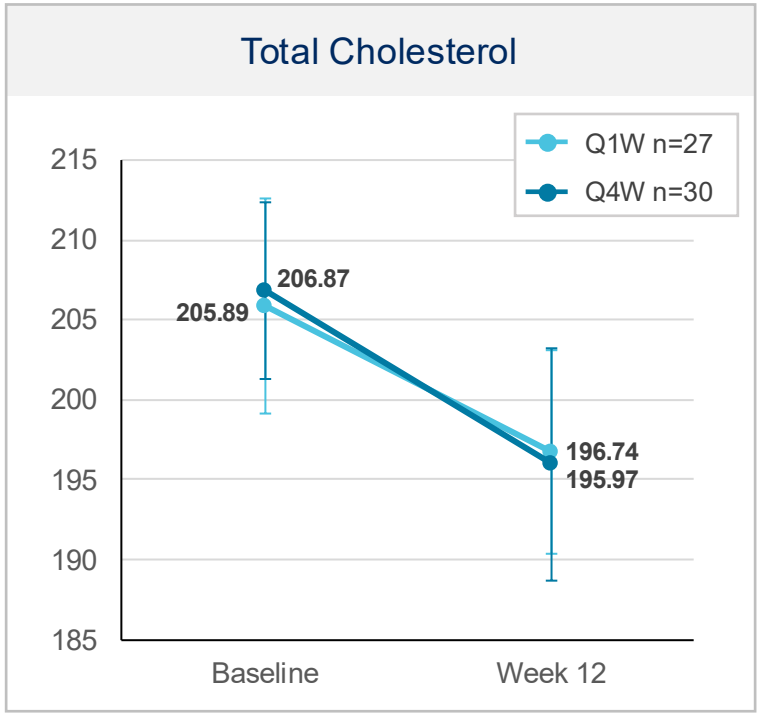
**Endpoints:** % change in total body weight, fat mass and lean mass at Week 24. Whole-body MRI in subset

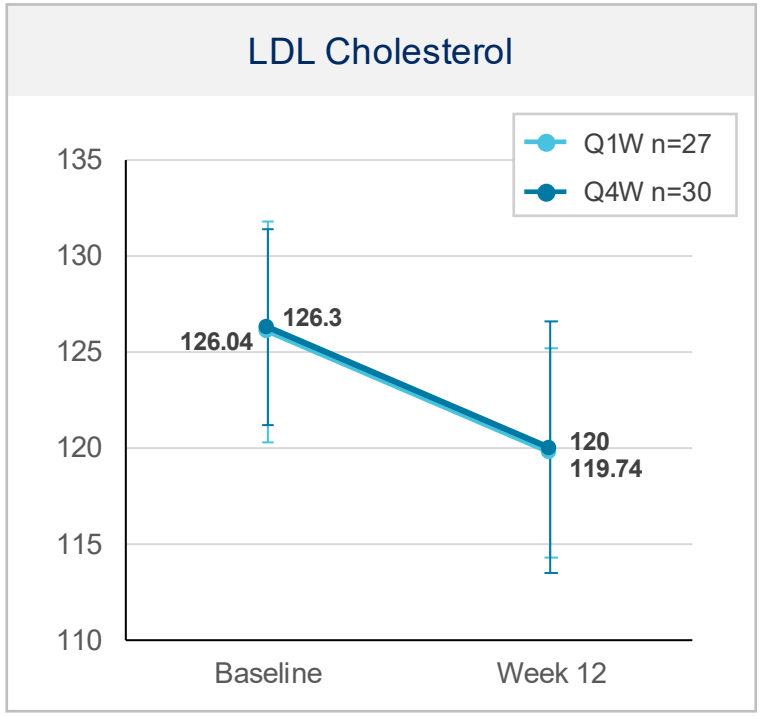
**KEY POINT**

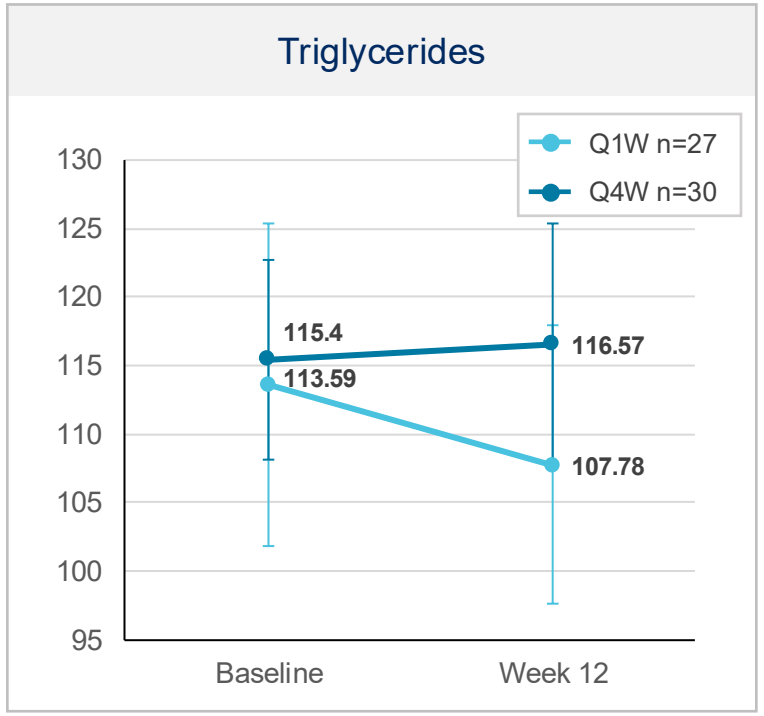
Planning to initiate Part B evaluating taldefgrobep + GLP-1 combination

# Blinded Interim Data by Q1W vs. Q4W, Taldefgrobep + Placebo
# No Adverse Effects on LIPIDS at Week 12

TALDEFGROBEP




- No treatment-emergent G2-4 elevations in total cholesterol or triglyceride values
- 1 participant with emergent G3 LDL elevation; had G2 elevation at baseline

Error bars represent standard error

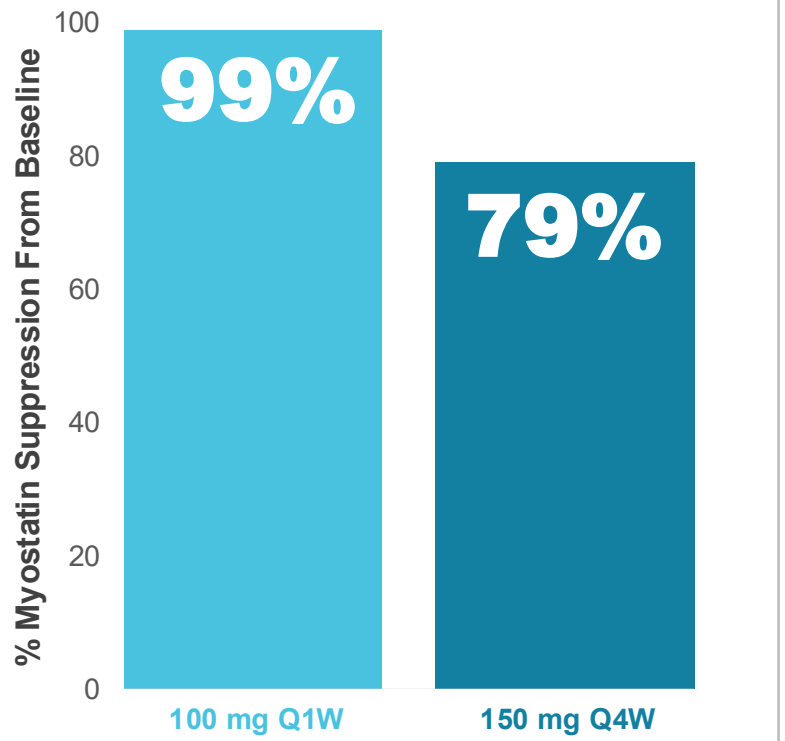
KEY POINT

No identified adverse trends in lipid parameters demonstrating differentiated safety from other myostatin-activin pathway inhibitors

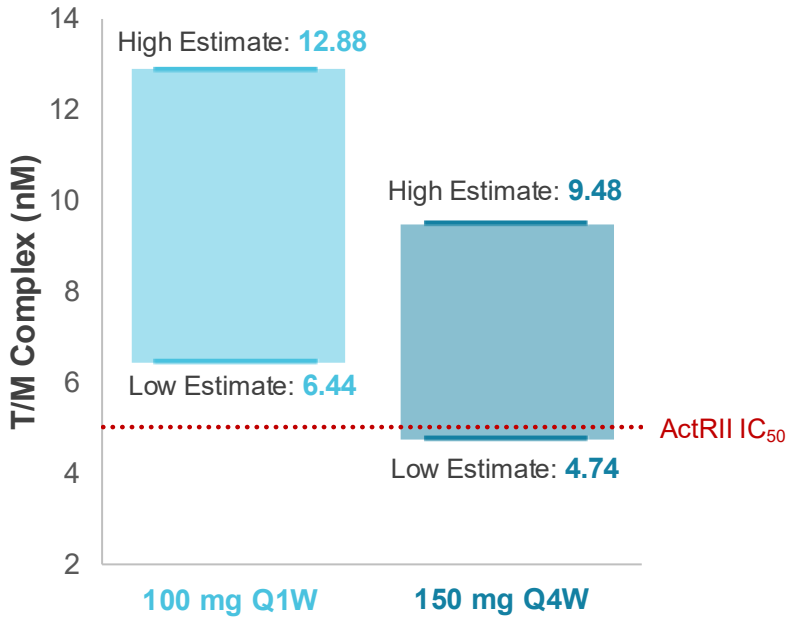
# Interim Phase 2 PK/PD Data Supports Weekly and Monthly Dosing in Obesity

TALDEFGROBEP

## % Myostatin Suppression at Week 12 in Overweight/Obese Adults




## Taldefgrobep-Myostatin Complex at Week 12 in Overweight/Obese Adults




## AT WEEK 12:

- Observed mean taldefgrobep concentrations are consistent with model-predicted
- Robust suppression of free myostatin throughout Q1W and Q4W dosing intervals
- T/M complex concentrations comparable between dosing regimens and at/above target ActRII $IC_{50}$

**KEY POINT**

Interim data suggest monthly dosing can achieve robust myostatin suppression and formation of T/M complex levels at/above targeted ActRII $IC_{50}$ levels

# Taldefgrobep Offers a Novel Approach To Address the Needs for People Living With Obesity

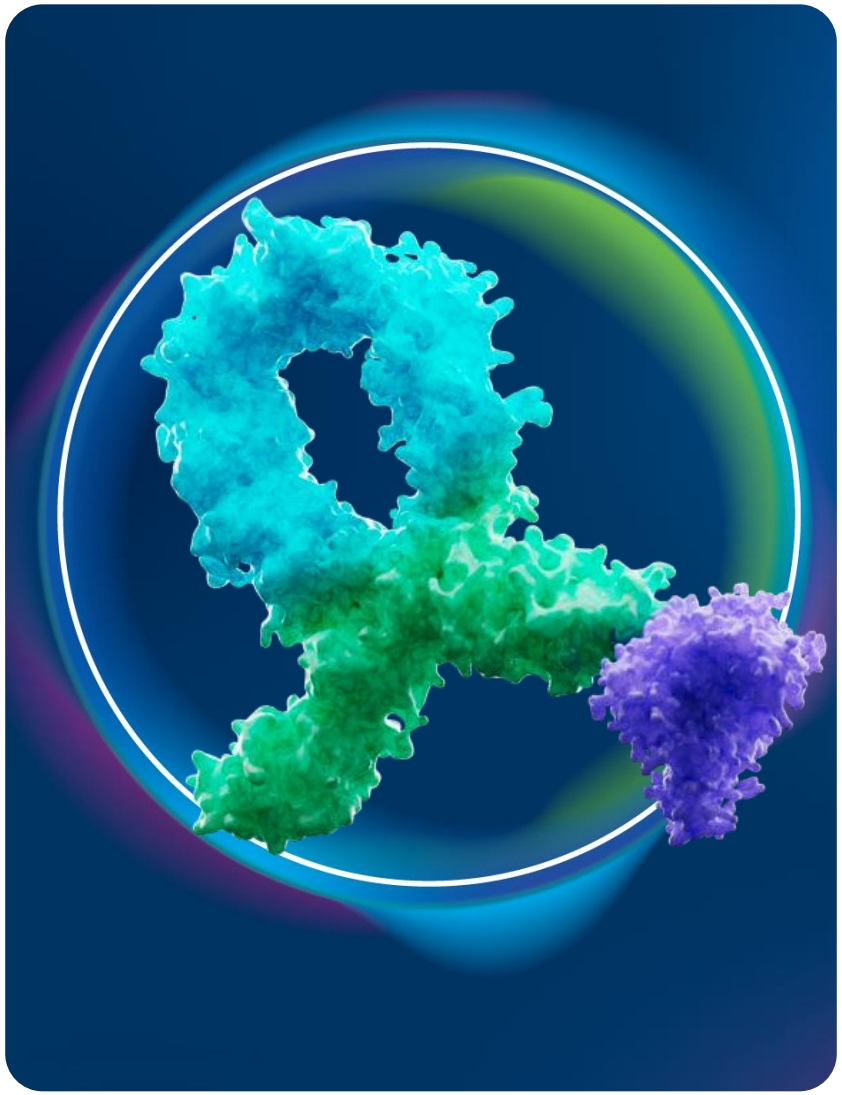
TALDEFGROBEP

**Total body weight loss** meeting current regulatory standards

**Benefit as monotherapy and in combination** with GLP-1 therapies

**Convenient** subcutaneous autoinjector with potential for monthly dosing

**Favorable safety and tolerability**



**Fat mass loss** comparable to GLP-1 therapies

**Visceral adipose tissue loss** favorable to GLP-1 therapies

**Increase in lean muscle mass** highly differentiated from GLP-1 therapies

**Increase in bone density** favorable to GLP-1 therapy

# Taldefgrobep Can Benefit a Broad Spectrum of People Living With Obesity

TALDEFGROBEP



**6.6M–10M**
Older patients at risk of sarcopenia

**5–8M**
People intolerant or refractory to GLP-1

**5–7M**
People living with BMI ≥40

**5–10M**
People unable to tolerate high dose GLP-1

MONOTHERAPY

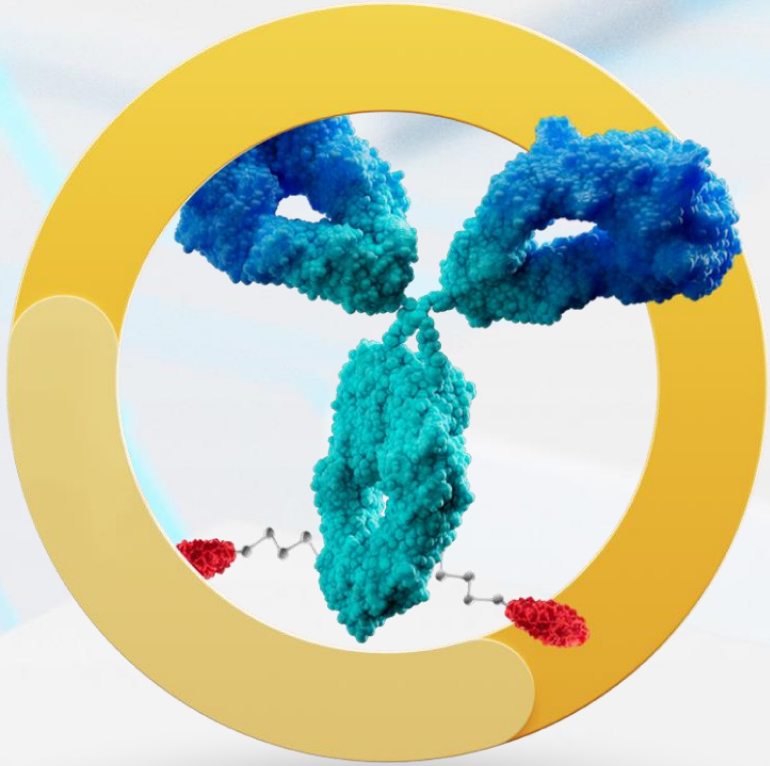
GLP-1 ADJUNCT

Source: BHVN Market Research and Analytics Data on file

KEY POINT

Meaningful reductions in fat mass, preservation of lean muscle mass, once-monthly dosing and high tolerability drive market potential

biohaven®

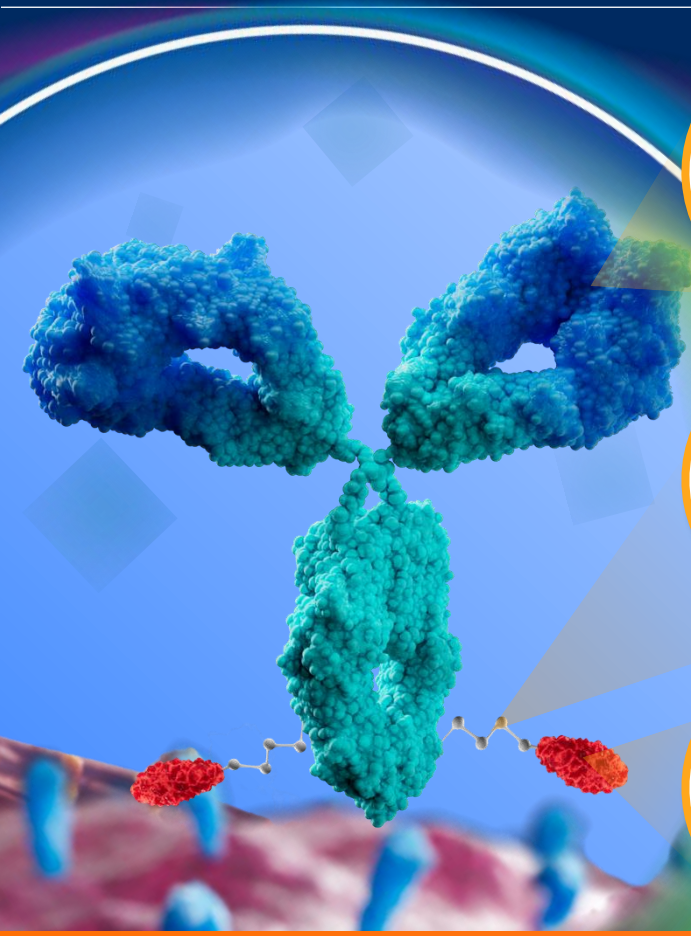


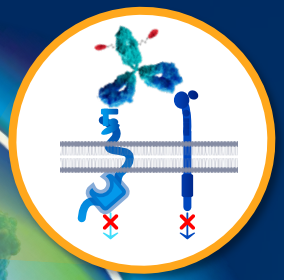
**ONCOLOGY**

# Modular Next-Generation ADC Technologies: Optimizing for Clinical Performance

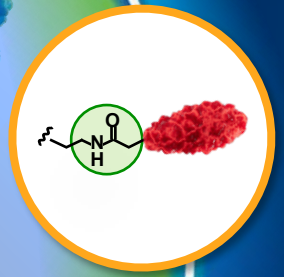
# Innovative Biohaven Technologies Enabling Differentiated Next-Generation ADCs

ONCOLOGY

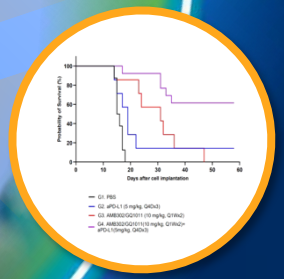




OPTIMIZED MONO AND BISPECIFIC MABS



NEXT-GEN MODULAR ADC CONJUGATION TECHNOLOGIES



PROPRIETARY TOPOIX AND MULTI-CLASS PAYLOADS

## Clinical Proof-of-Principle Establishing the Power and Flexibility of the Platform to Optimize Across a Broad Range of ADC Designs and Combination Strategies Including CPI

**First clinical demonstration (BHV-1510 Trop2)**
Highly stable, differentiated PK and safety, compelling activity with CPI combination

**Pioneering FGFR3 ADC (BHV-1530)**
Novel mAb, potential to address both FGFR3 alterations and overexpression

**Platform tech enables multi-class payload loading**
Clinically differentiated TopoIx payload, foundational for CPI and other potentially synergistic MOA combinations

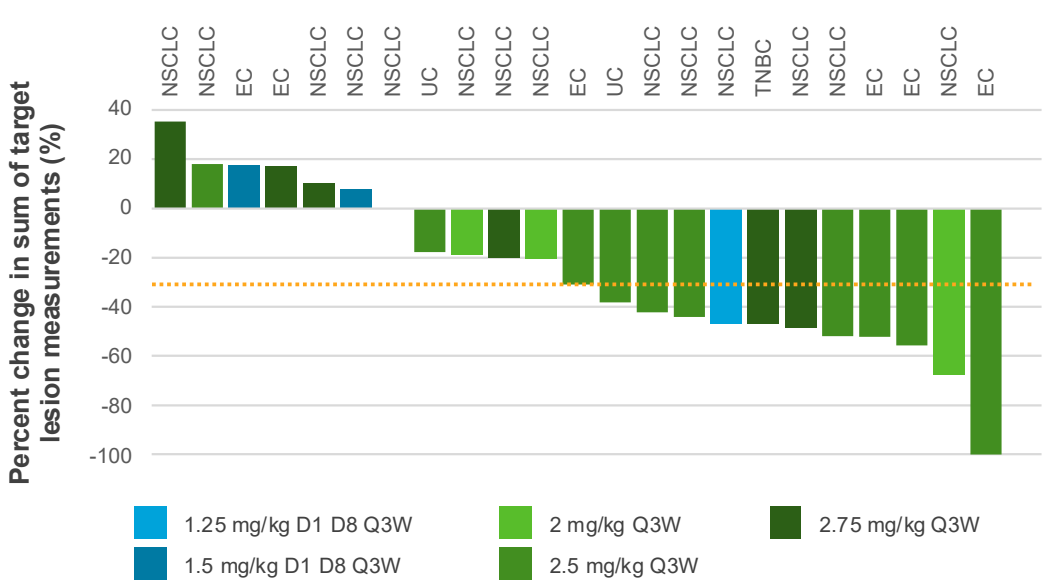
BREAKING NEWS

- **BHV-1510:** Robust enrollment in endometrial + cemiplimab combination expansion cohort
- **BHV-1530:** First activity observed, including pretreated FGFR3-altered urothelial cancer patient; combination cohort with Libtayo now enrolling under clinical supply agreement with Regeneron.
- **Discovery:** Preclinical data supporting next-wave bispecific mAb, multi-payload formats

# BHV-1510 + Cemiplimab Leads to Rapid, Deep and Durable Responses in Heavily Pretreated Patients, Majority With Prior Anti-PD(L)1

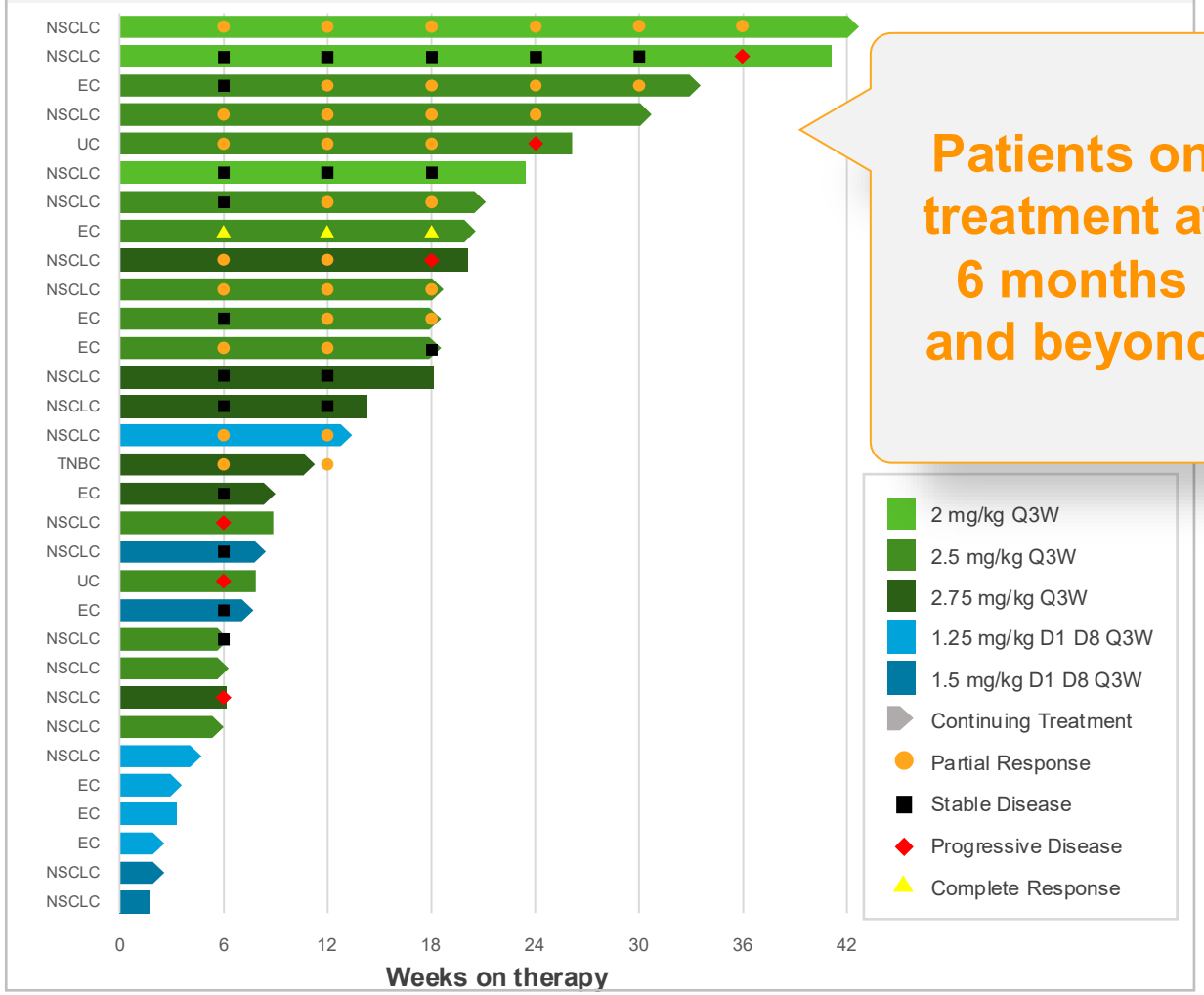
ONCOLOGY

## BHV-1510 + Cemiplimab*: Percent Change in Target Lesions




**Majority (87.1%) had prior anti-PD(L)1 exposure**

## BHV-1510 + Cemiplimab: Treatment Duration




**Patients on treatment at 6 months and beyond**

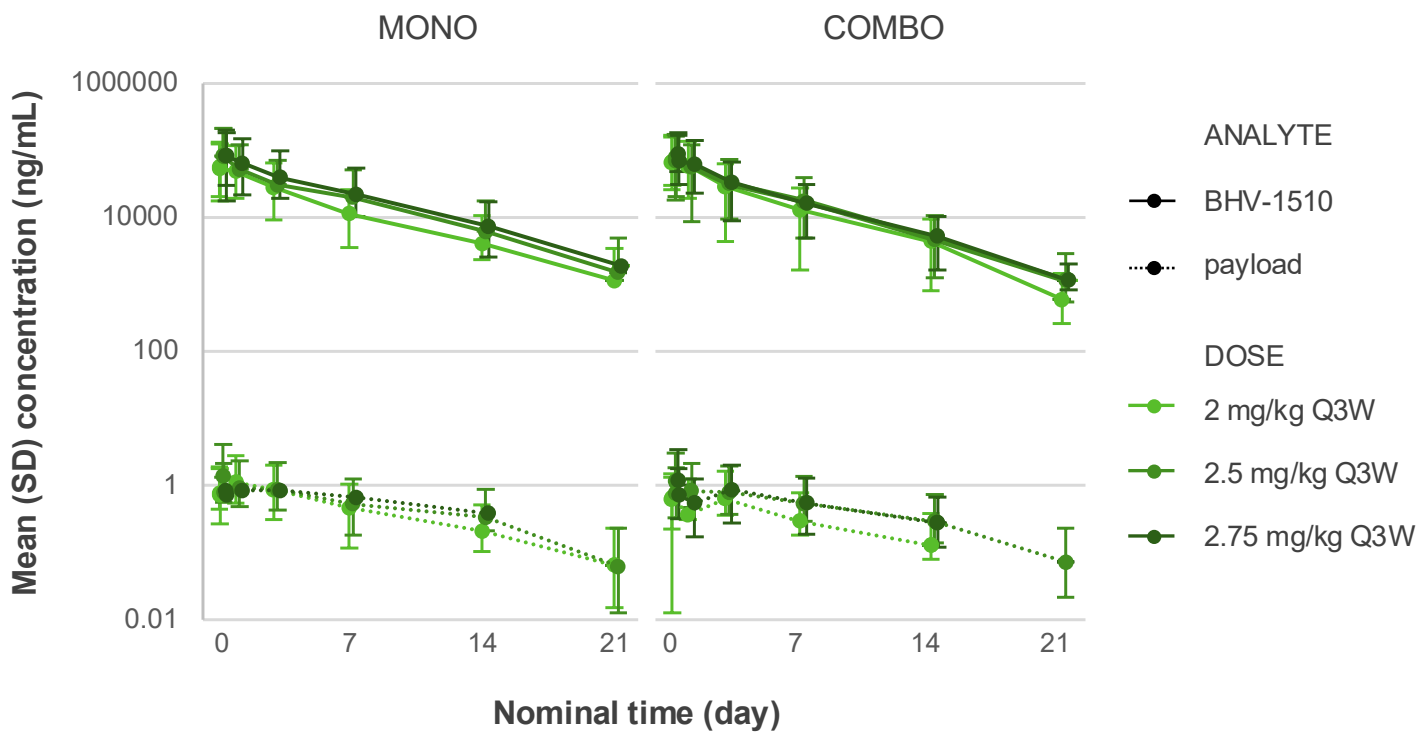
*Cemiplimab (anti-PD1) provided through a supply agreement with Regeneron; dose of cemiplimab 350 mg Q3W

**Source:** Micaily. ESMO Immuno-Oncology Congress 2025. Poster 252P.

# BHV-1510 Demonstrates a Favorable PK Profile With Highly Stable ADC

ONCOLOGY

## BHV-1510 (ADC) and Payload Concentration-Time Plots for Cycle 1 (with or without Cemiplimab)




**Source:** Micaily. ESMO Immuno-Oncology Congress 2025. Poster 252P.

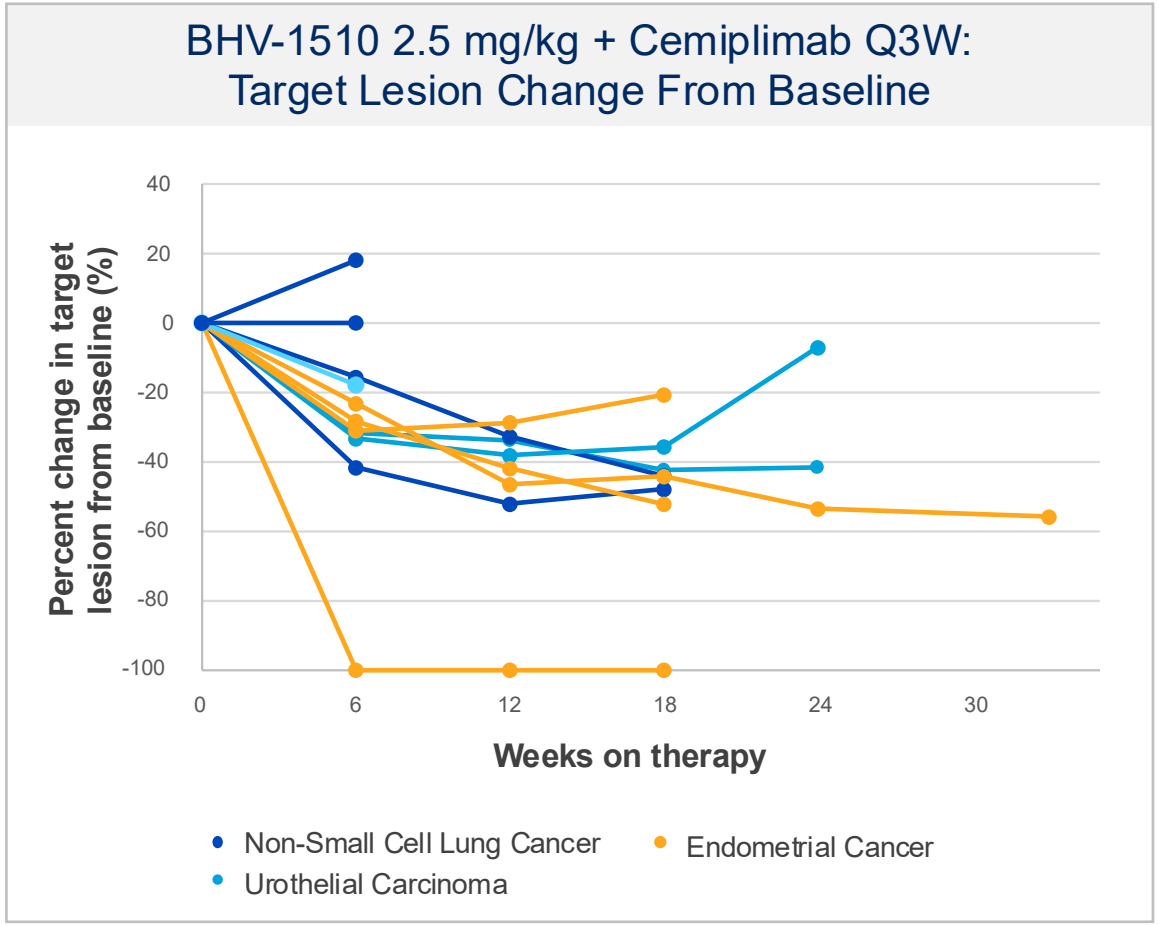
**KEY POINT**

The unconjugated payload concentration was low with a payload-to-ADC molar ratio <1%, indicating that ADC was highly stable in the circulation

# Encouraging Early Clinical Activity and High Response Rates of BHV-1510 + Cemiplimab Enable First Tumor-Specific Expansions

ONCOLOGY

- **Dose escalation complete:** Compelling efficacy in difficult-to-treat patients
  - Responses in heavily pretreated patients, including with brain metastases; majority with prior anti-PD(L)1 exposure
- **Rapid onset of benefit:** Tumor shrinkage / PRs at 1st scan
- **Differentiated safety profile:** Low rates of hematological toxicities and diarrhea; no ILD observed*
- **Favorable PK profile:** Highly Stable ADC




* By independent adjudication

**Source:** Micaily. ESMO Immuno-Oncology Congress 2025. Poster 252P.

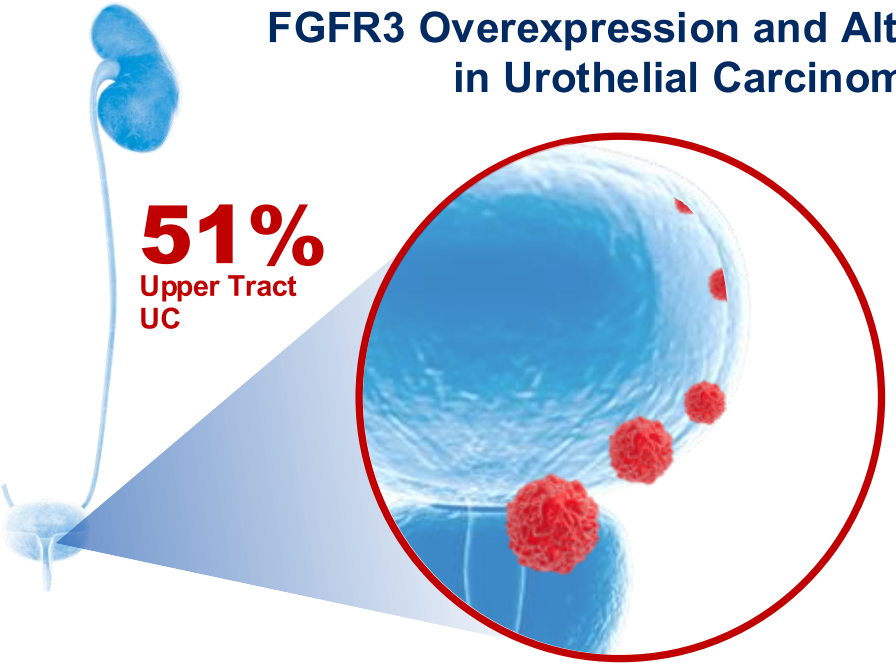
KEY POINTS

- Early data suggests synergy with anti-PD-1 and potential to move into earlier lines
- Endometrial cancer expansion cohort with combination enrolling robustly — data to inform pivotal path

# BHV-1530: Favorable Early Clinical Profile — No FGFR-Class Toxicities and Early Signs of Antitumor Activity

ONCOLOGY

## FGFR3 Overexpression and Alteration (%) in Urothelial Carcinoma



**51%**
Upper Tract UC

**70–80%**
Non-muscle-invasive BC

**40–60%**
Muscle-invasive BC

**~50%**
Metastatic UC

**FGFR3**

**~50%** of HNSCC

**~ 40%** of NSCLC

**4–8% alterations** in other cancers

As of April 2026. Data from ongoing study

**Compelling preclinical efficacy across FGFR3-altered and FGFR3-overexpressing tumor models:** Demonstrated as monotherapy and in combination with CPI

**Clinical progress:** First patient dosed April 2025: initial cohorts successfully completed:

- No dose limiting toxicities
- No treatment-related SAEs
- TRAEs predominantly mild (Grade 1–2)
- No hyperphosphatemia, nail disorders, central serous retinopathy

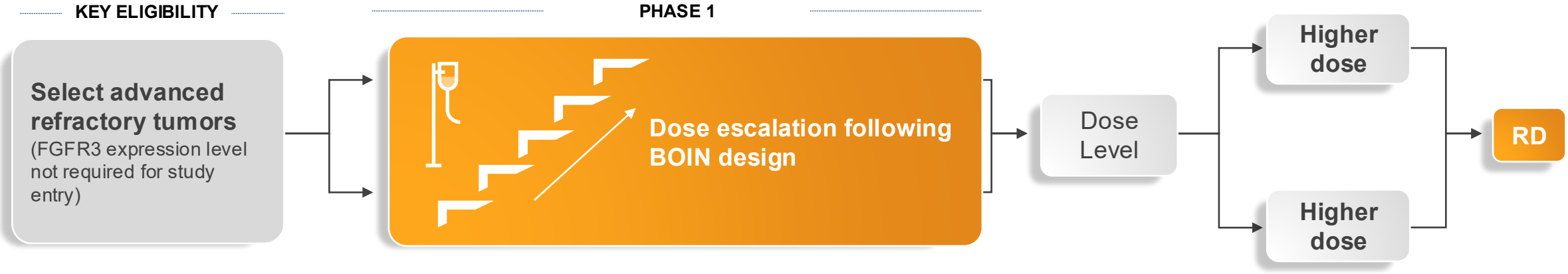
**Early signs of activity:** Early tumor reduction in patients with FGFR3 alterations and wild-type overexpression, as dosing in the predicted efficacious range

**KEY POINTS**

- Combination cohort with Libtayo now enrolling under clinical supply agreement with Regeneron
- Early tumor reductions in FGFR3-altered and WT overexpressing patients, including urothelial cancer
- Dose escalation continuing with no DLTs to date

# BHV-1530: Phase 1 Study in Advanced Tumors

ONCOLOGY




## KEY STUDY DETAILS

**Study Design:** Open label, dose escalation (Ph1)

**Population:** Advanced UC, HNSCC, NSCLC having failed SOC therapy

**Treatment Duration:** Until disease progression or toxicity

**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.

**KEY POINT**

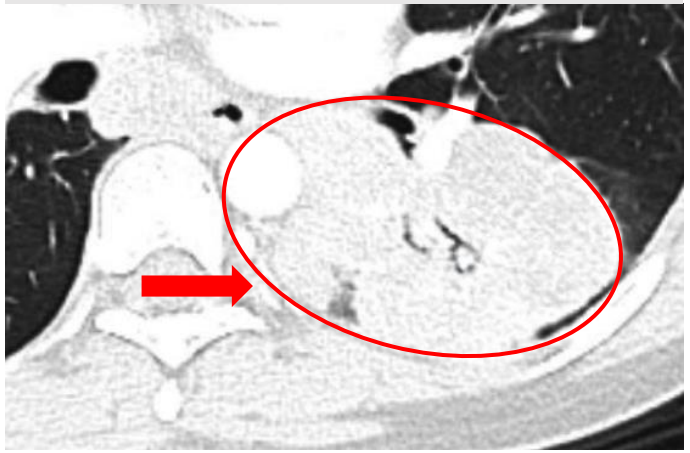
Early tumor reduction in patients as dosing in the predicted efficacious range

# Case Narrative: Metastatic Urothelial Cancer

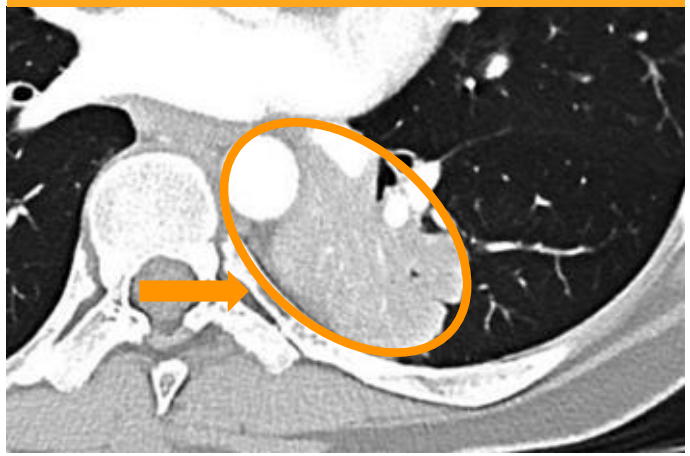
ONCOLOGY

- 28 y/o female with urothelial cancer with a FGFR3::TACC3 fusion, with metastasis to lungs and thoracic lymph node
- 4 prior lines of therapy including
  - Padcev and Keytruda and 2 FGFR targeting small molecules-Balversa and LOXO-435
- Tolerating treatment with mild nausea and no FGFR related toxicity

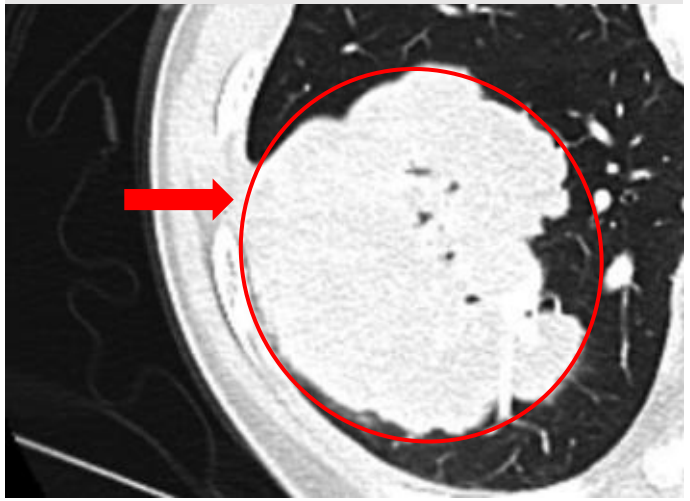
Baseline



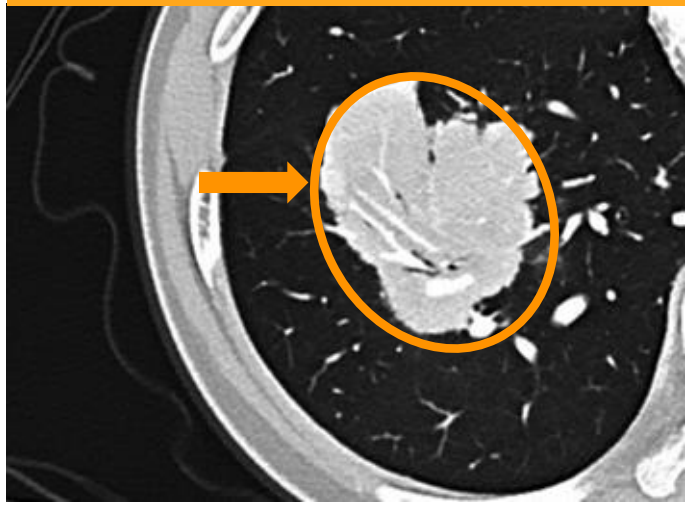
Week 12



Baseline



Week 12



# BHV-1530: Potential First FGFR3 ADC — Broader Reach, No Class Toxicities, Clear Development Path

## BHV-1530 ADC vs. Small-Molecule FGFR3 Inhibitor

| | Small Molecule Inhibitor | BHV-1530 |
|---|---|---|
| **Target Population** | FGFR3-altered only<br>~limited addressable population | **Altered AND overexpressing — full FGFR3+ population** |
| **Class Toxicities** | Hyperphosphatemia<br>nail disorders, retinopathy | **None observed — no FGFR inhibitor-class toxicities** |
| **Mechanism** | Kinase inhibition<br>resistance develops readily | **ADC: targeted delivery of cytotoxic payload** |
| **CPI Combination** | Limited — overlapping toxicity concerns | **Engineered for CPI synergy**<br>**TopoIx → ICD →** |

**3x larger TAM in mUC (15-20% are FGFR3 altered and up to 50% are altered/or overexpressed)**

## DEVELOPMENT ROADMAP

### PH1 DOSE ESCALATION
NOW • ACTIVE
- No DLTs; no FGFR-class toxicities
- Early tumor reduction in altered + WT overexpressing

### DOSE OPTIMIZATION+POC
- Identify recommended Ph2 dose
- UC expansion POC
- Confirm activity across FGFR3 subtypes

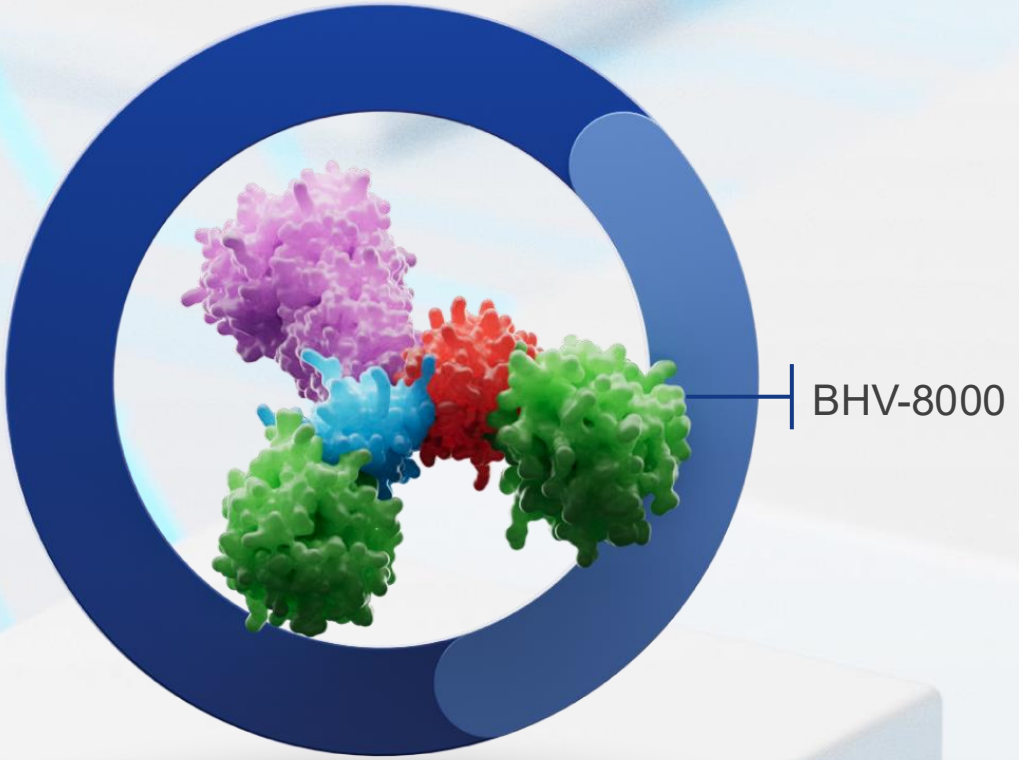
### COMBO EXPANSION
- Expansion cohort in UC with CPI combo
- Pivotal design conversations begin

### 1L mUC - PIVOTAL PATH
- 1L mUC combination with CPI
- Monotherapy in 2L+-selected pts

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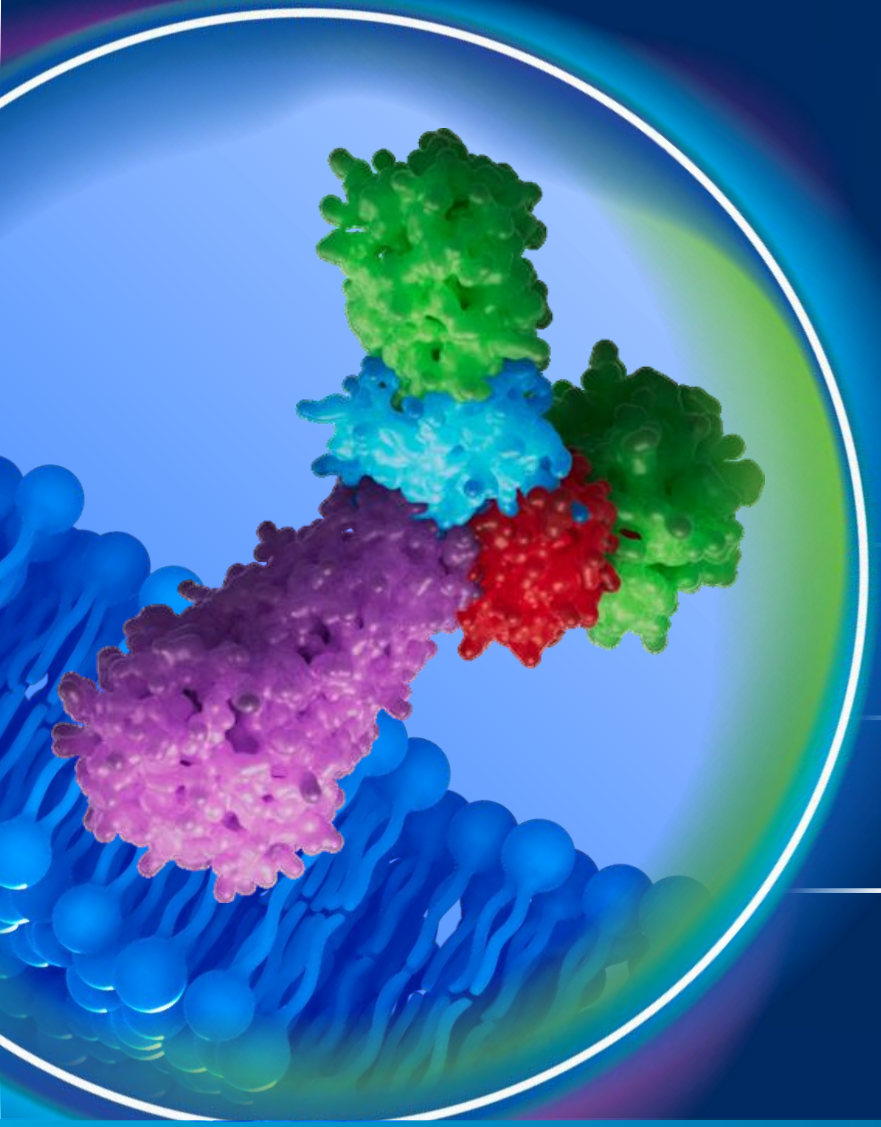
**BRAIN-PENETRANT TYK2/JAK1 INHIBITOR: BHV-8000**

# Leveraging Validated Immune Targets To Stop Neuroinflammation



# Brain-Penetrant TYK2/JAK1 Inhibitor To Treat Parkinson's Disease

TYK2/JAK1



**BHV-8000 addresses the widespread immune dysregulation that drives the onset and progression of neurodegenerative disorders**

**Suppresses innate and adaptive immune cell activation and infiltration**

**High selectivity for TYK2/JAK1 avoids JAK2/3 safety liabilities**

**Demonstrated target engagement, robust brain penetration and a promising safety profile**

**Phase 2/3 trial in early-stage PD ongoing**

# Widespread Immune Dysregulation Is a Hallmark of PD

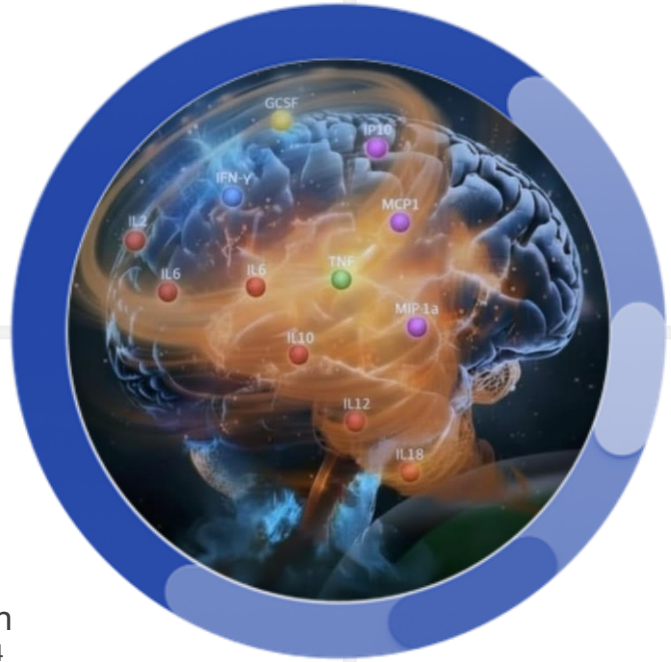
TYK2/JAK1

## 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]

## 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]




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

- Epidemiological 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]

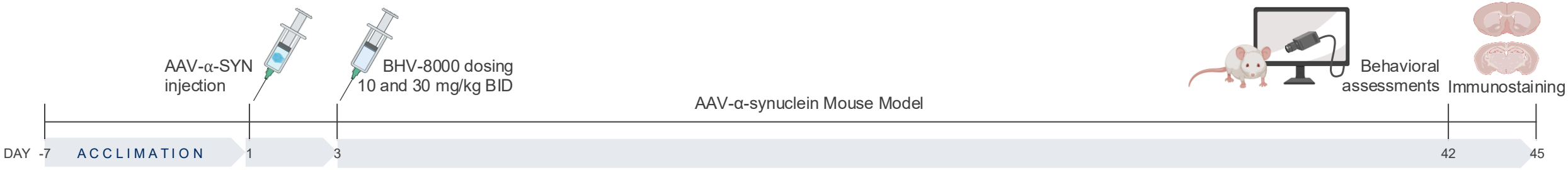
## PD patient samples and imaging exhibit characteristic proinflammatory signatures[9,10]

- 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]

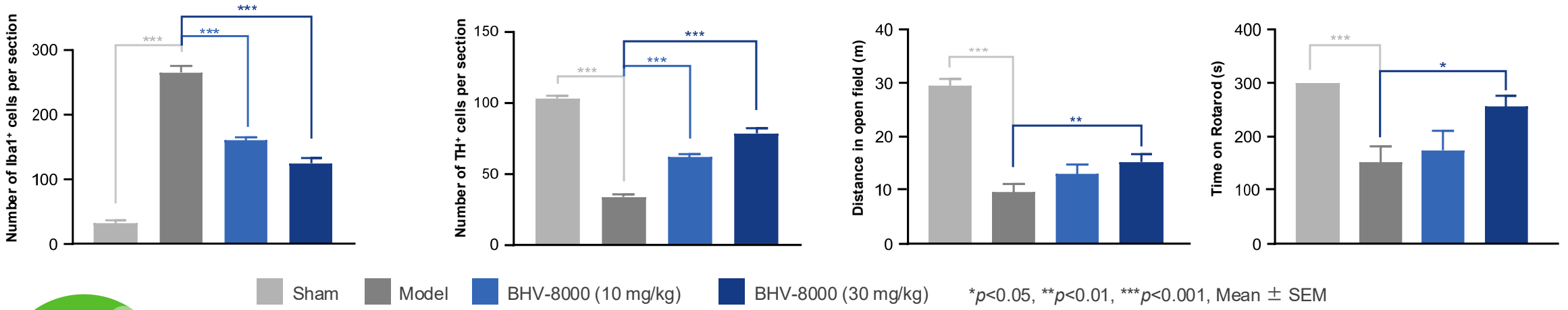
**1.** Sulzer. Nature. 2017. **2.** Wissemann. Am J Hum Genet. 2013. **3.** Li. Front Immunol. 2023. **4.** Tansey. Nat Rev Immunol. 2022. **5.** Potashman. Parkinsonism Relat Disord. 2025. **6.** Roodveldt. Brain. 2024. **7.** Karikari. Brain Behav Immun. 2022. **8.** Williams. Brain. 2021. **9.** Yacoubian.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

# BHV-8000 Reduces Glial Activation, Increases Neuronal Survival, and Improves Function in AAV-α-syn Mouse Model of Parkinson's Disease

TYK2/JAK1




| Reduced Inflammation | Increased Neuronal Survival | Improved Function | |
|---|---|---|---|
| Reduced the Number of Activated Glial Cells (Iba1+) in the Substantia Nigra: | Increased the Survival of TH+ Neurons in the Substantia Nigra: | Increased Movement Distance in an Open Field by: | Increased Time on Rotarod by: |




**KEY POINT**

BHV-8000 reduces inflammation and improves neurological function

# BHV-8000 Demonstrates Target Engagement, Robust Brain Penetration and a Promising Safety Profile in Phase 1 Studies

TYK2/JAK1

## 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

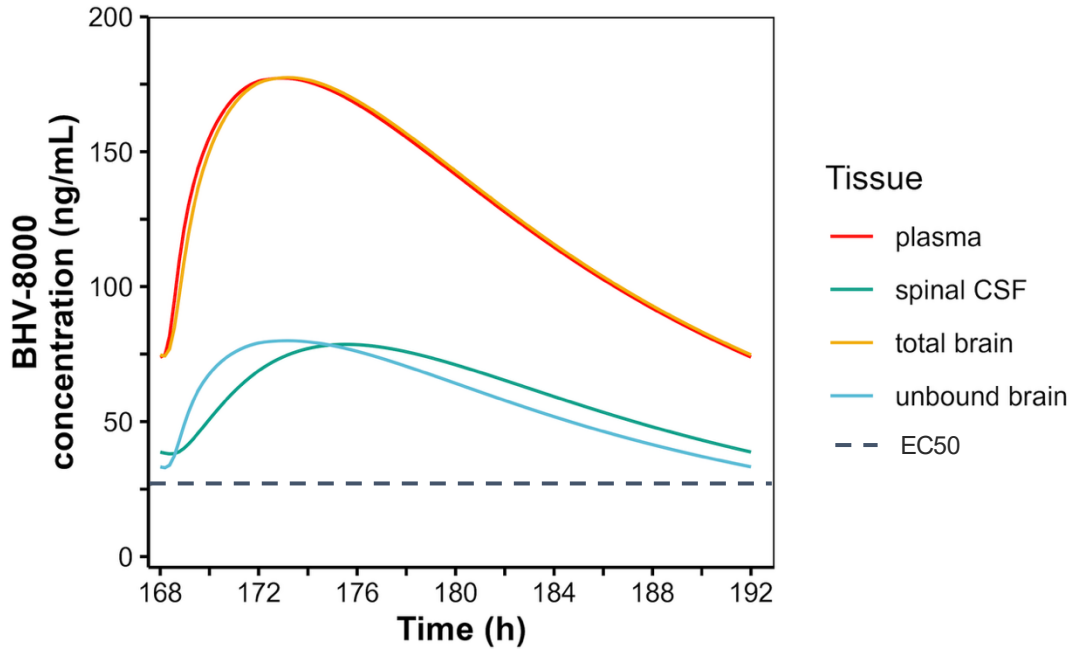
## Evidence of target engagement

- 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



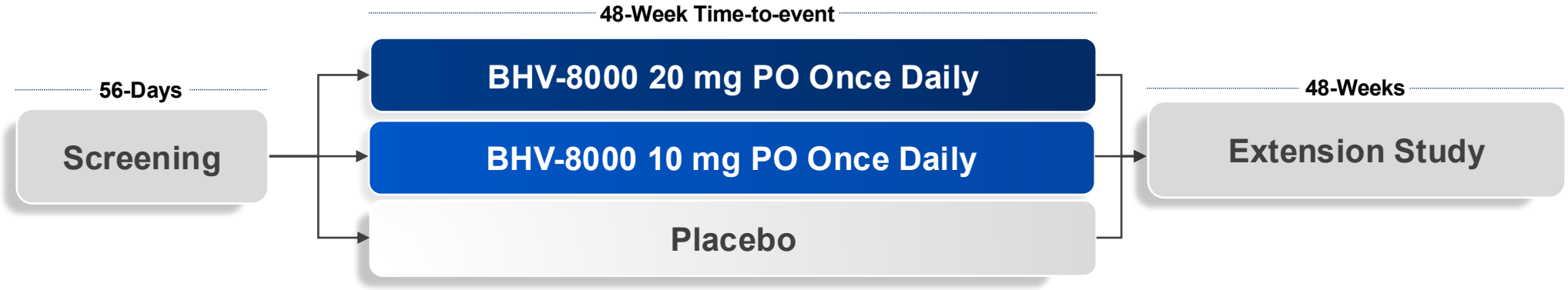

Modeling data

KEY POINT

Brain exposure sustained above target EC50s for 24 hours at clinical doses

# BHV-8000: Phase 2/3 Study in Early Parkinson's Disease Ongoing

TYK2/JAK1




**KEY STUDY DETAILS**

**Study Design:** Phase 2/3, randomized 1:1:1, double-blind study

**Population:** Early untreated PD

**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

**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 design enables efficient trial

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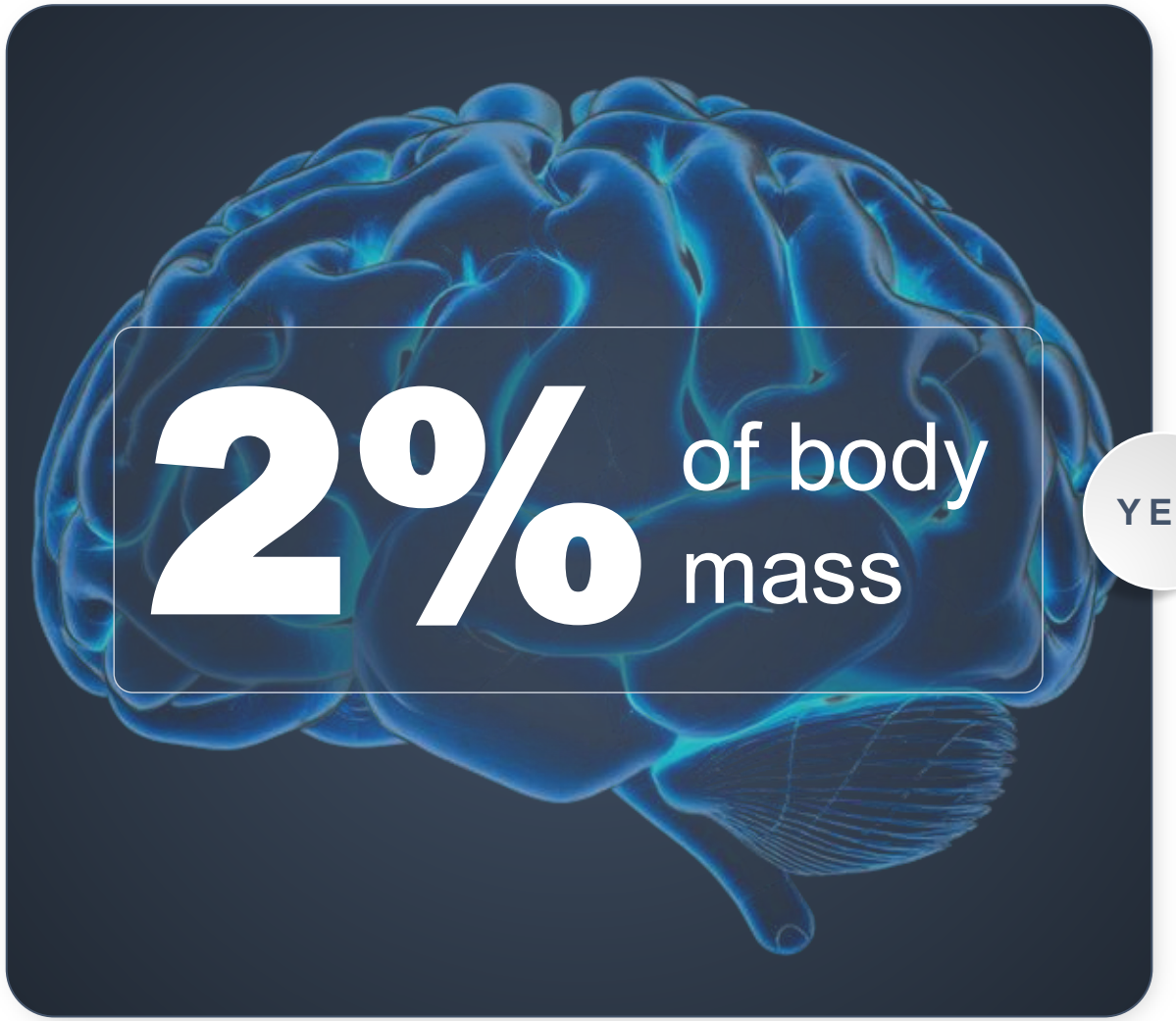
**BRAIN-PENETRANT PKM2 ACTIVATOR:**
**BHV-8100**

DAYS MATTER™

# Targeting Brain Hypometabolism to Treat Neurodegenerative Diseases

# The Brain Has Considerable Energetic Requirements

DISCOVERY




YET

**15%** of **cardiac output**

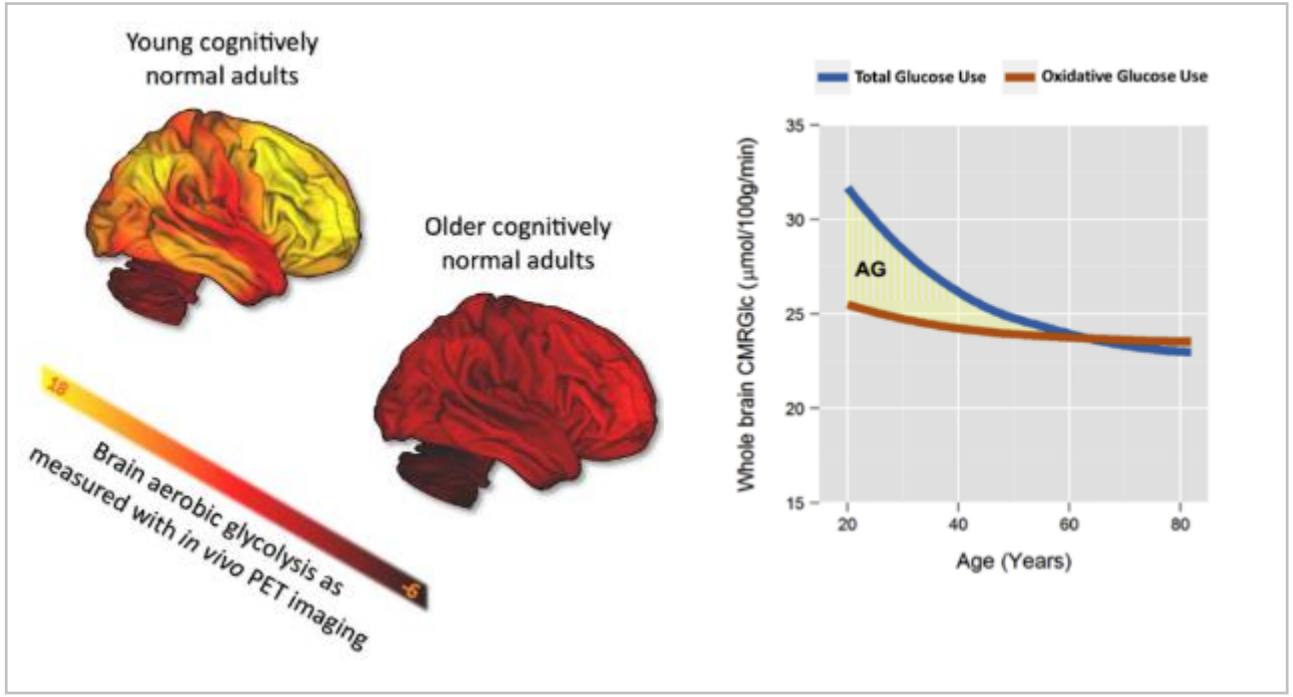
**25%** of whole-body **glucose utilization**

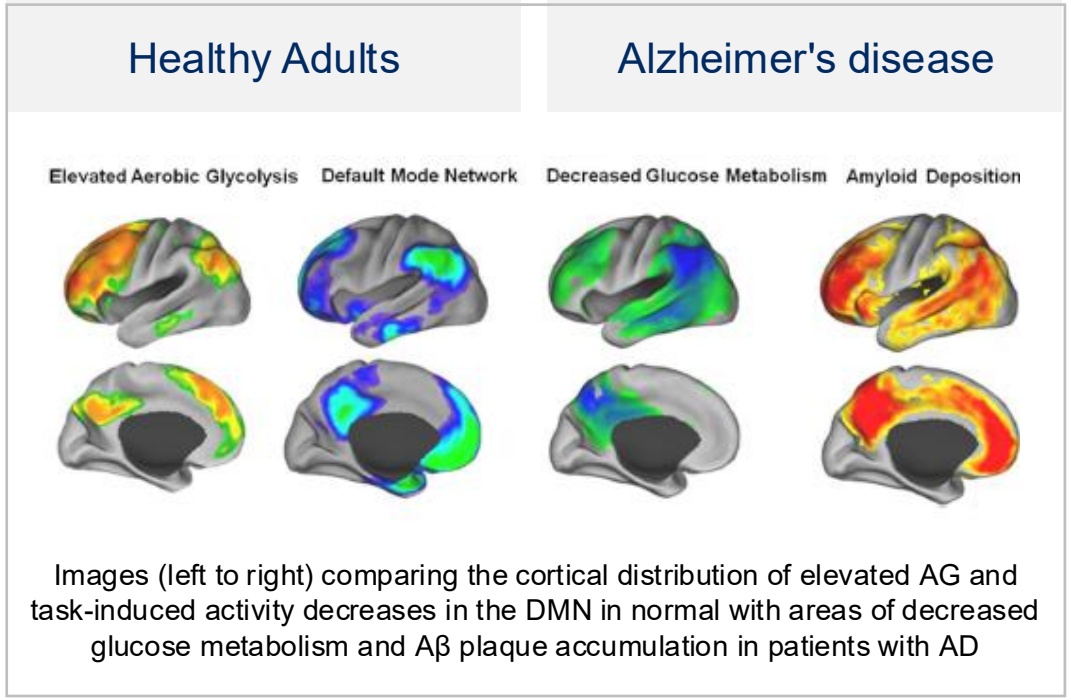
**20%** of **oxygen consumption**

# Aerobic Glycolysis Is Reduced in the Aging Brain

DISCOVERY

- **Aerobic glycolysis**, the metabolic process of glucose primarily promoted by astrocytes in the brain, is reduced in the aging brain[1]
- Reduction of aerobic glycolysis is pronounced in frontal and temporal regions[2]



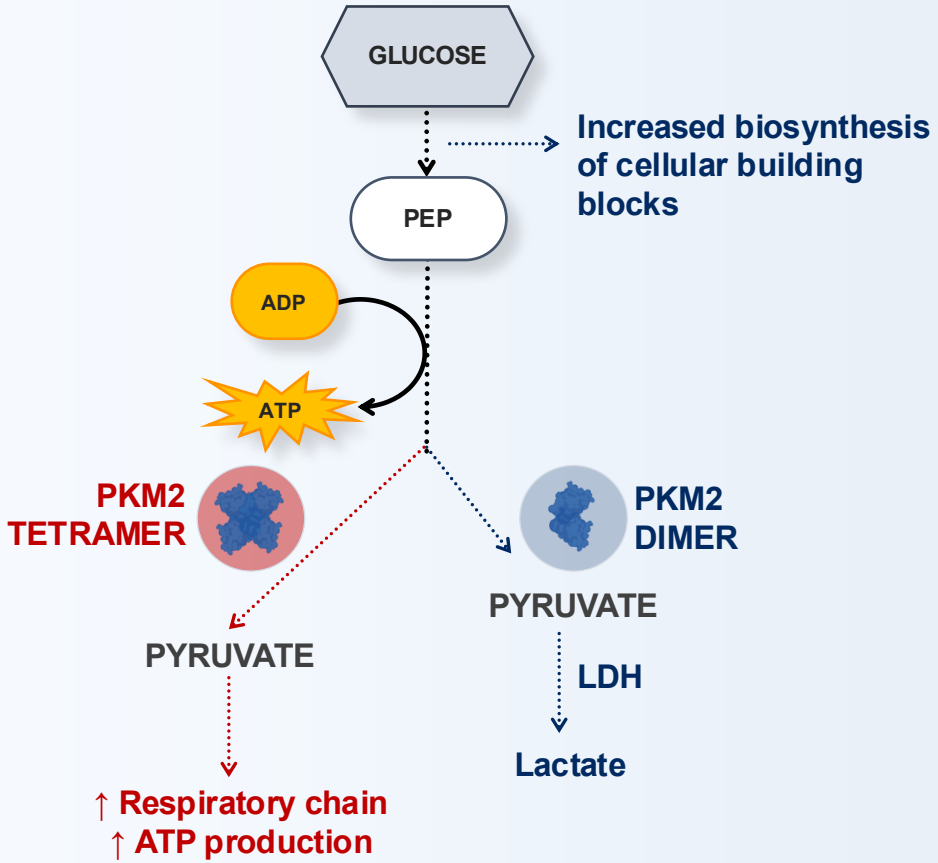



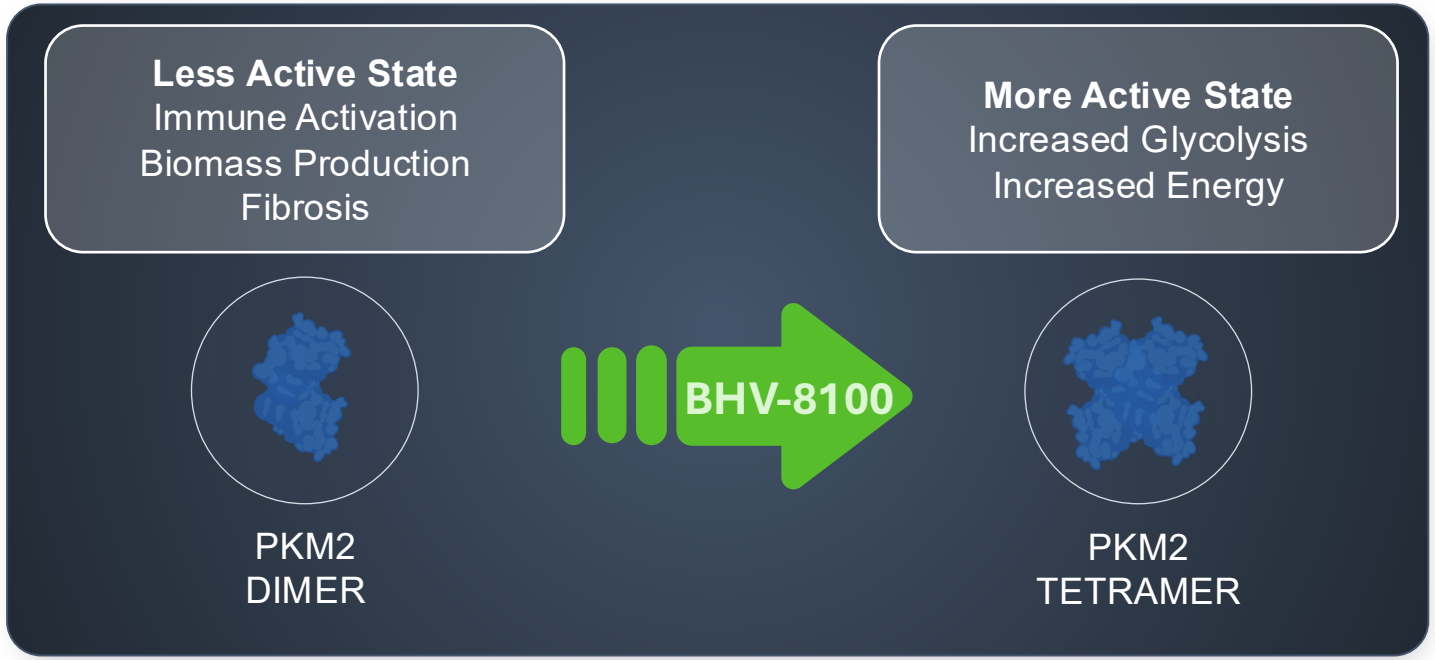

Images (left to right) comparing the cortical distribution of elevated AG and task-induced activity decreases in the DMN in normal with areas of decreased glucose metabolism and Aβ plaque accumulation in patients with AD

**1.** Goyal. Cell Metab. 2017. **2.** Vlassenko. Clin Transl Imaging. 2015.

# Activation of Pyruvate Kinase Increases Glucose Consumption and ATP Production While Reducing Deleterious Metabolism

DISCOVERY



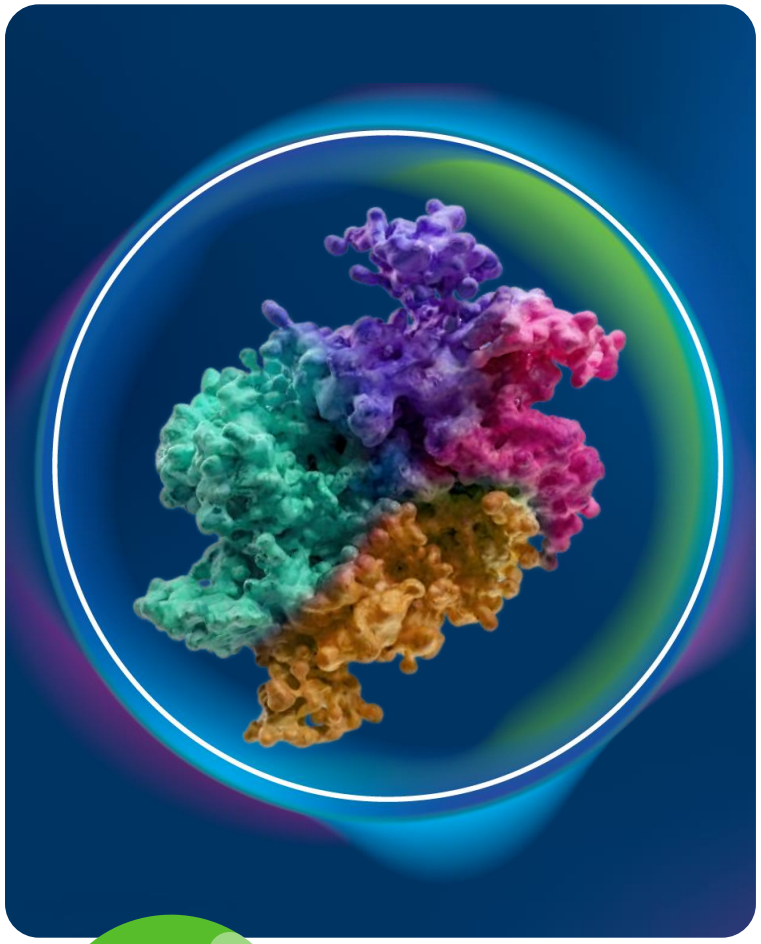




- BHV-8100 stabilizes enzymatically active tetramer state
- Increased energy in CNS
- Decreased neuroinflammation, angiogenesis and fibrosis
- Reduced deleterious metabolic intermediates

**Source:** Cancer Letters. 2015.

# BHV-8100: First Brain-Penetrant PKM2 Activator

DISCOVERY



## Oral small molecule medicine with multiple potential indications

- Neurodegeneration
- Retinal degeneration and inflammation
- Neuroinflammation
- Age-related changes: central/peripheral
- Brain ischemia
- Peripheral inflammation

**KEY POINT**

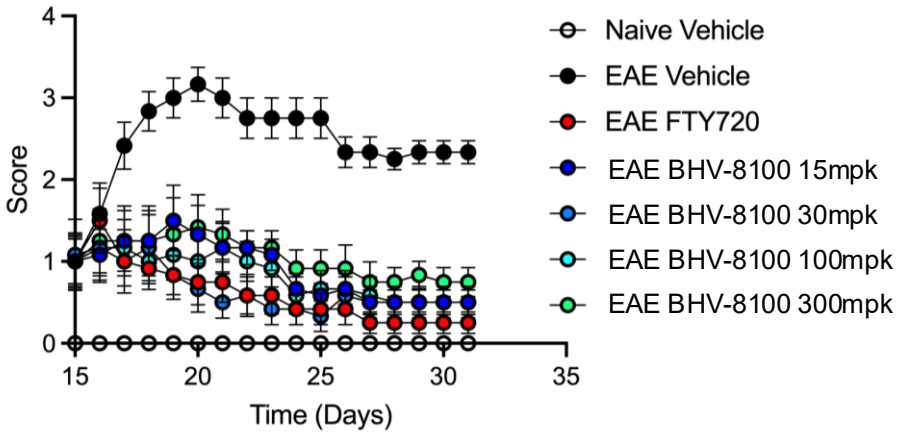
First-in-human dosing at pharmacologically relevant doses initiated 2Q 2026

# BHV-8100: Experimental Mitigation of Neuroinflammation and Restoration of Energetic Deficiency

DISCOVERY

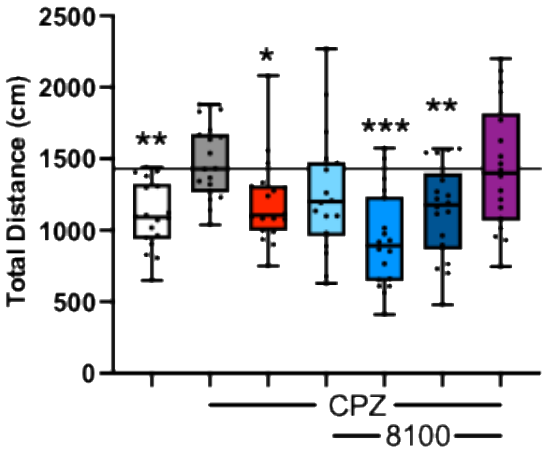
## BHV-8100 Shows Robust Reduction of Disease Burden in EAE

Clinical Score (EAE Therapeutic Model)

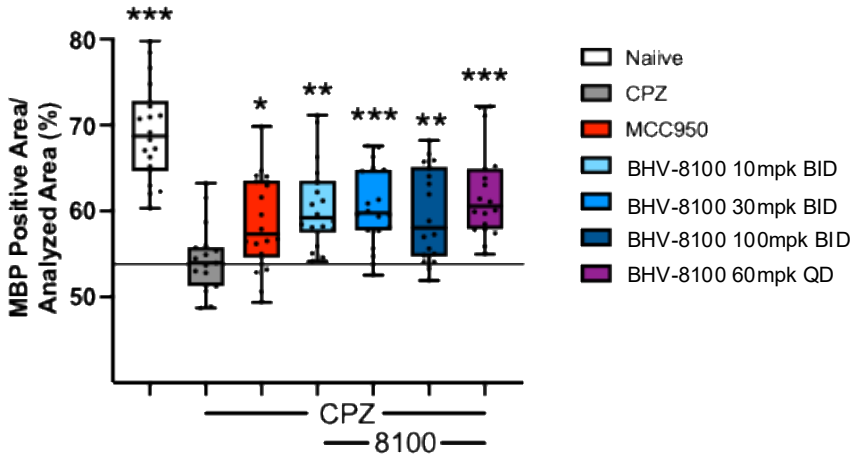



## BHV-8100 Demonstrates Striking Efficacy in Cuprizone Model

Distance Traveled




Myelin-Basic Protein in Corpus Callosum




* p < 0.05, ** p < 0.01, *** p < 0.001 relative to CPZ group, MCC950 is an NLRP3 inhibitor.

KEY POINT

BHV-8100 demonstrates anti-inflammatory efficacy and a strong ability to promote oligodendroglial function in models of EAE

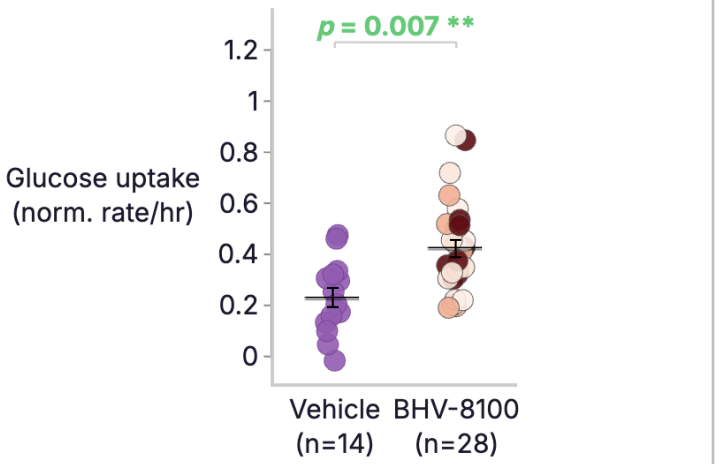
# BHV-8100 Demonstrates Sustained Efficacy in Human Brains With Documented Alzheimer's Disease and All-cause Dementia

DISCOVERY

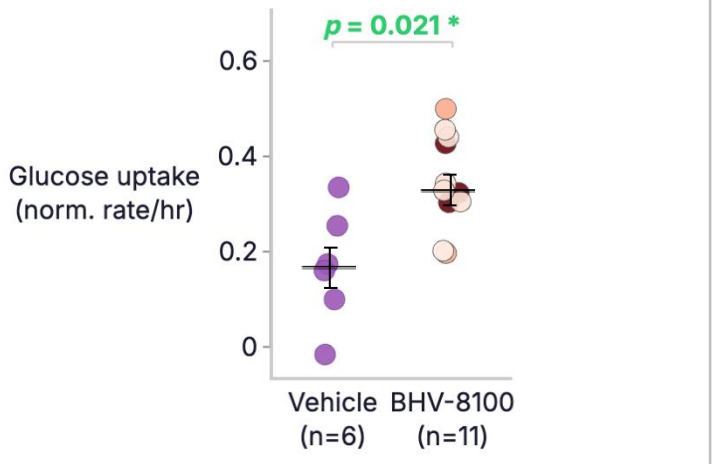
Reperfused human brains (Brainex™) allow precise study of brain penetrance, pharmacology, pharmacokinetics, pharmacodynamics and biomarkers in brains with documented diseases



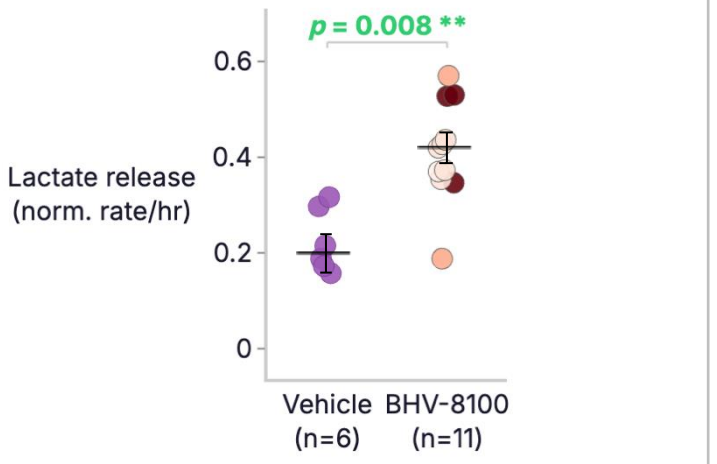

## Disease of Cognitive Impairment[1]: BHV-8100 Enhances Glucose Uptake




## Alzheimer's Disease: BHV-8100 Enhances Glucose Uptake




## Alzheimer's Disease: BHV-8100 Enhances Lactate Production




1. Diverse diagnoses including AD and other disease.

KEY POINT

Precise determinations of brain penetrance and dose-response pharmacology confirm efficacy and guide human dosage

# Early FIH Data of BHV-8100 in SAD Shows Safety and Tolerability in Healthy Participants

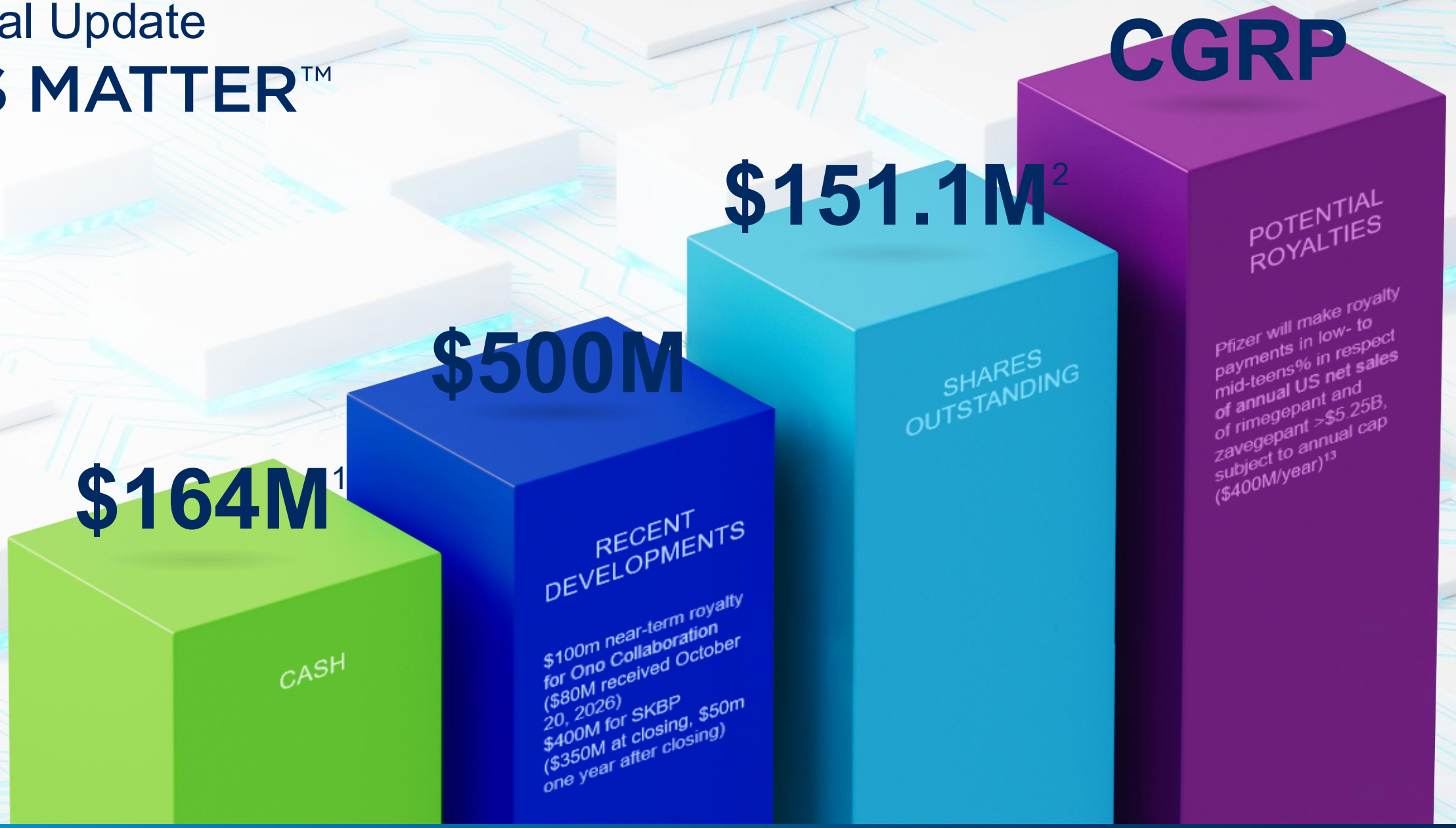
DISCOVERY

- No SAEs or severe AEs
- Most AEs mild and spontaneously resolving
- No clinically significant ECG changes
- No clinically significant trends in safety labs including LFTs
- PK allows QD dosing & achieves projected efficacious exposures

Preliminary data from ongoing Phase 1 Study BHV8100-101 (as of 29-May-2026)

# Financial Update

# DAYS MATTER™




**1.** Includes cash, cash equivalents and marketable securities as of September 30, 2026; **2.** As of October 26, 2026. **3.** Cap reached if aggregate annual US 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.

# Key Milestones Anticipated in 2026 - 2027

| | | | 2H 2026 | 1H 2027 | 2H 2027 |
|---|---|---|---|---|---|
| GLUTAMATE | **Troriluzole \| BHV-4157** | Spinocerebellar Ataxia | NDA Resubmission Accepted | AdCom<br>PDUFA Target Action Date | |
| INFLAMMATION & IMMUNOLOGY | **Gd-IgA1 Degrader \| BHV-1400** | IgA Nephropathy | Initiate Pivotal IgAN Trial | | |
| | **IgG Degrader \| BHV-1300** | Common Disease (Graves', RA) | Ongoing Pivotal Graves' Trial | | |
| | **TYK2/JAK1 Inhibitor \| BHV-8000 (brain-penetrant)** | Parkinson's Disease | Ongoing Phase 2/3 Trial | | |
| MYOSTATIN ACTIVIN | **Taldefgrobep Alfa \| BHV-2000** | Obesity | | | Phase 2 Topline<br>(inc GLP-1 Combo) |
| ION CHANNEL | **Kv7 Activator \| Opakalim** | Focal Epilepsy | Pivotal Topline | | |
| ONCOLOGY | **Trop2 ADC +/- PD-1 \| BHV-1510** | Advanced or Metastatic Epithelial Tumors | Ongoing Expansion Cohort in Endometrial Cancer | | |
| | **FGFR3 ADC +/- PD-1 \| BHV-1530** | Urothelial Cancer and Other Tumors | Ongoing Phase 1 in Urothelial Cancer | | |

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