

biohaven<sup>®</sup>

DAYS  
MATTER™

43rd Annual J.P. Morgan  
Healthcare Conference

January 13, 2025

**Vlad Coric, M.D.**

Chairman and Chief Executive Officer

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BHVN  
LISTED  
NYSE



**JENNIFER**  
Living with SCA3

Participant in the  
Troriluzole Clinical Study

# Forward-Looking Statement

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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 clinical trials for our taldefgrobep alfa, troriluzole, BHV-2100, BHV-7000, BHV-8000, BHV-1300, BHV-1310, BHV-1510 and BHV-1530 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-1400, BHV-1500, and BHV-1600. 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; the timing and outcome of expected regulatory filings; complying with applicable US regulatory requirements; the potential commercialization of Biohaven's product candidates; the potential for Biohaven’s product candidates to be first-in-class or best-in-class therapies; and the effectiveness and safety of Biohaven's product candidates. 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 is 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**  
into top areas of  
**INNOVATION**

IMMUNOLOGY & INFLAMMATION

NEUROLOGY

OBESITY

ONCOLOGY

CARDIOVASCULAR

RENAL

RARE DISEASE

Focused on Days Matter™  
**COMMERCIALIZATION  
OF NOVEL TXs**

**INCREASED  
MARKET CAP**

**10X**

POSITIONED FOR  
**FUTURE  
VALUE  
CREATION**

**ADVANCING  
CANCER  
TREATMENTS**  
with strategic partnerships

**TWO YEARS  
SINCE SPIN-OFF**

**biohaven**®

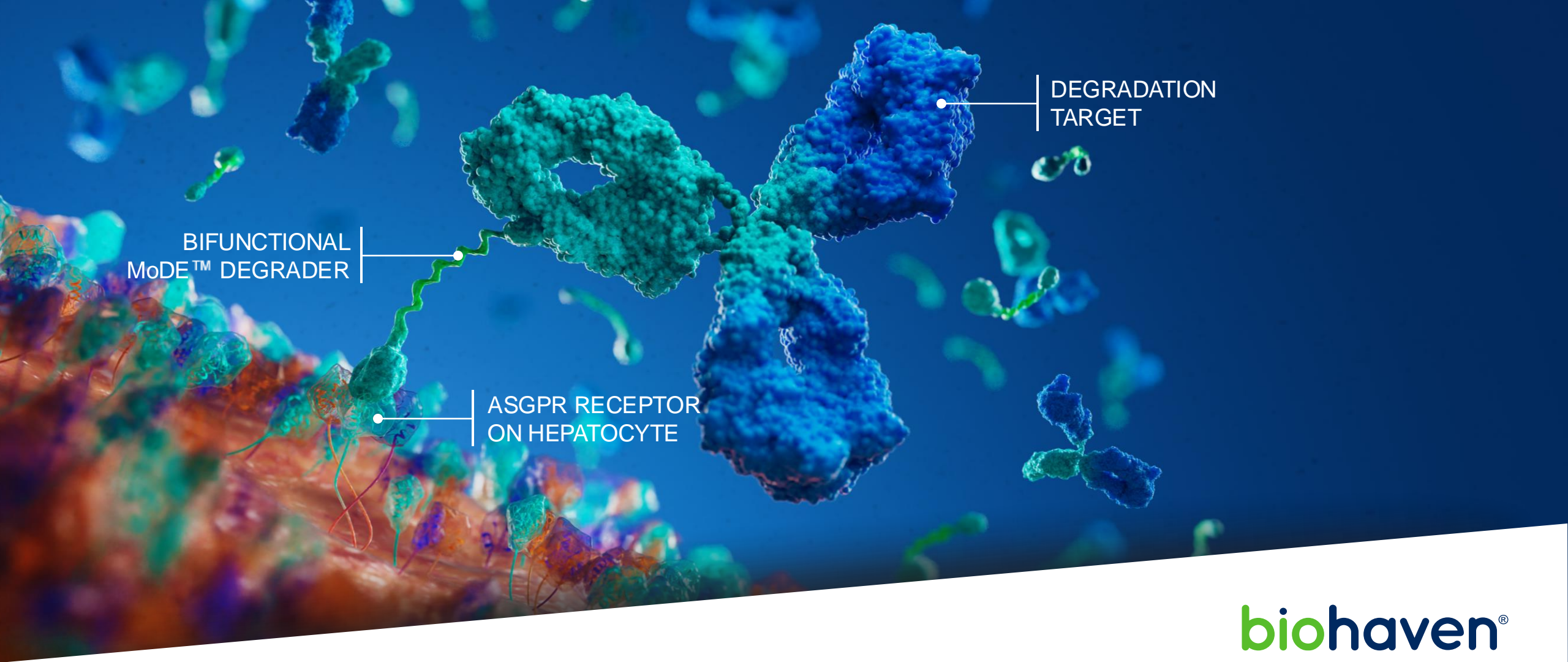
**PIONEERING**  
therapies for  
rare diseases  
including  
**SMA &  
SCA**

**CLINICALLY  
VALIDATED  
NEXT-GEN TRAP™  
DEGRADERS**

Targeted removal of  
aberrant proteins

Integrated  
**DISCOVERY  
ENGINE**





DEGRADATION  
TARGET

BIFUNCTIONAL  
MoDE™ DEGRADER

ASGPR RECEPTOR  
ON HEPATOCYTE

biohaven®

# EXTRACELLULAR DEGRADERS

RAPID AND SELECTIVE REMOVAL OF DISEASE-CAUSING PROTEINS

## MoDE™ Platform: Degraders Designed for Real-life and to Preserve Healthy Immune Functioning

- Maximizes selectivity to treat disease while minimizing side effects
- Short half-life enables concomitant administration with Fc-biologics
- Allows for subcutaneous and autoinjector formulations

## Advancing Next-Generation TRAP™ (Targeted Removal of Aberrant Proteins) Degraders:

- Only degrades specific disease-causing targets while leaving healthy immune system completely intact
- New Phase 1 clinical trial data demonstrates deep, rapid, and selective lowering of very specific targeted species

## 3 Exciting New Indications

IgA Nephropathy | Peripartum Cardiomyopathy | Graves' Disease



# DEGRADERS

BREAKING  
NEWS

Emerging clinical data with BHV-1400 shows rapid, deep, and selective removal of only galactose-deficient IgA1 while preserving healthy immune function

# MoDE™ Degradation Platform Technology: Driving Toward Targeted Removal of Disease-Causing Proteins

## Next-Gen TRAP™ Degradation

MoDE  
Degradation  
Platform



IgG DEGRADER  
Graves' disease

IgG DEGRADER  
Rheumatoid  
arthritis (RA)

IgG DEGRADER  
Myasthenia  
gravis (MG)

Gd-IgA1 DEGRADER  
IgA Nephropathy

$\beta_1$ AR AUTOANTIBODY  
DEGRADER  
Peripartum  
cardiomyopathy  
(PPCM)

Gd-IgA1 DEGRADER  
IgA Vasculitis

$\beta_1$ AR AUTOANTIBODY  
DEGRADER  
Dilated cardiomyopathy  
(DCM)

IgG4 DEGRADER

- Membranous nephropathy (MN)
- Pemphigus vulgaris
- Autoimmune encephalitis (AE)
- Muscle-specific kinase (MuSK) myasthenia gravis (MG)
- Chronic inflammatory demyelinating polyneuropathy (CIDP)

PROINSULIN  
AUTOANTIBODY DEGRADER  
Diabetes

TSHR  
AUTOANTIBODY  
DEGRADER  
Graves'  
disease

PLA2R  
AUTOANTIBODY  
DEGRADER  
Membranous  
nephropathy  
(MN)

Other  
indications

## Degrader Platform Technology

### FAST AND DEEP

Removes disease-causing proteins within hours

### EASY-TO-USE

- Easy-to-use autoinjector for self-administration
- Allows for concomitant use of biologics

PATIENT CENTRIC



LIFE ALTERING

### SELECTIVE

Designed to target specific pathogenic species for maximal efficacy and minimal side effects

### TUNABLE

- Level of degradation carefully modulated by dose level and frequency
- Employs body's natural mechanism for removal of senescent proteins

biohaven®

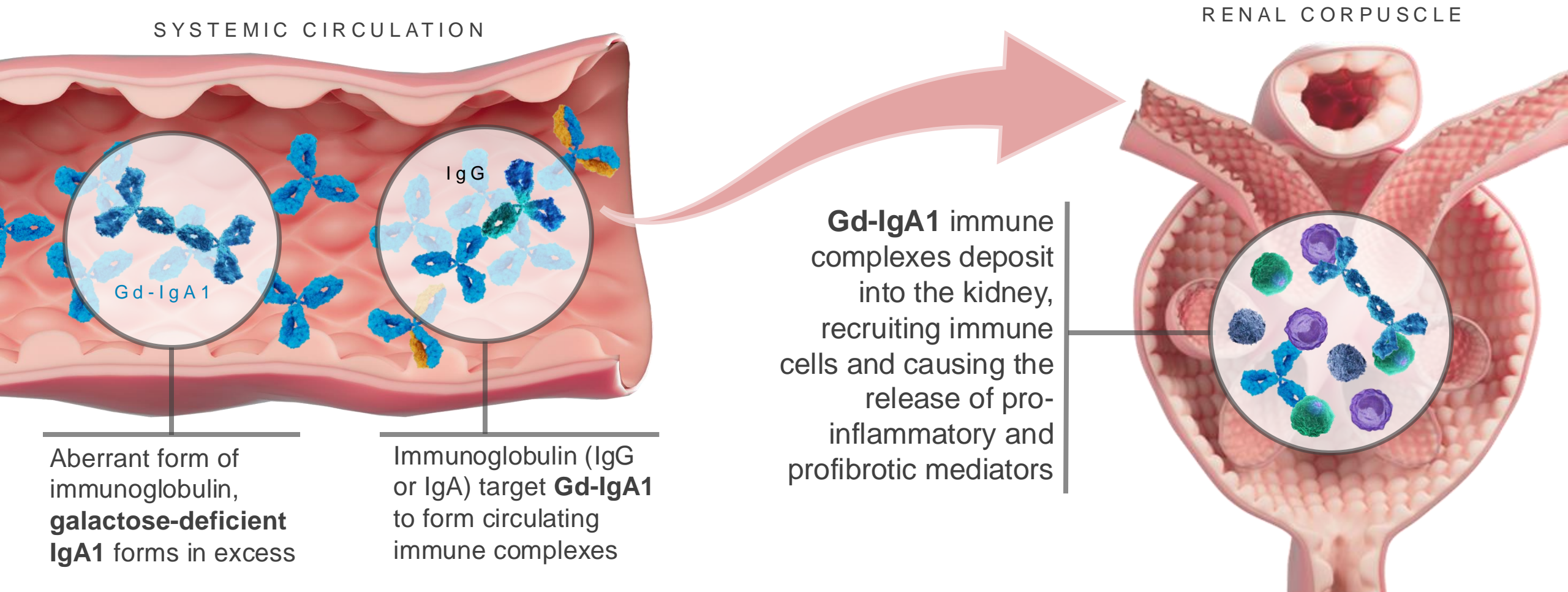




- ✓ BHV-1400: Potential to treat by removing pathogenic species without chronic immunosuppression
- ✓ Robust science indicating disease is galactose-deficient IgA1-driven
- ✓ Biomarker endpoint with well-established accelerated approval pathway

# IgA NEPHROPATHY

# 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**

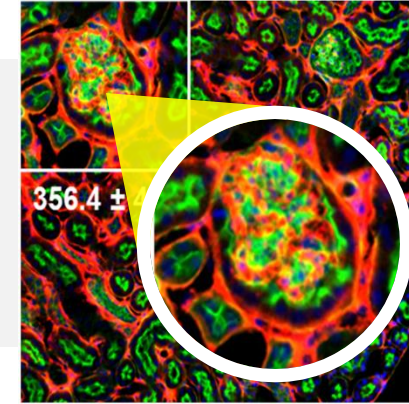
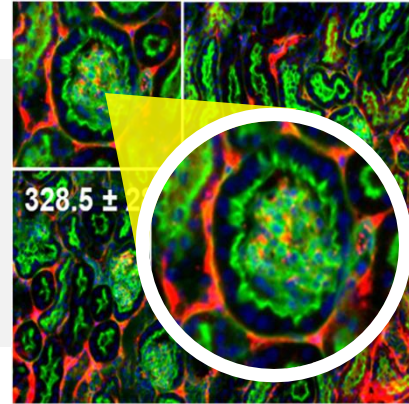
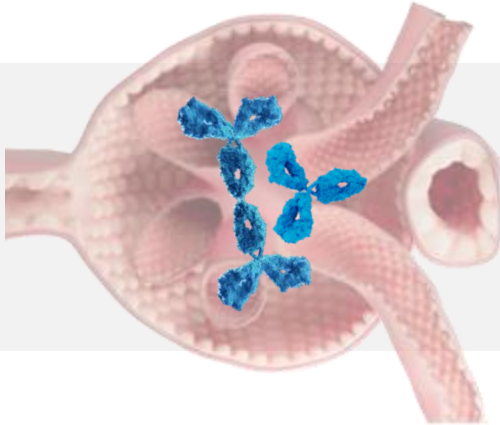
# BHV-1400 Rapidly Removes Galactose-Deficient IgA1 from Circulation and from the Renal Glomerular Mesangium *in vivo* in Pre-Clinical Studies

RENAL CORPUSCLE

1 HOUR

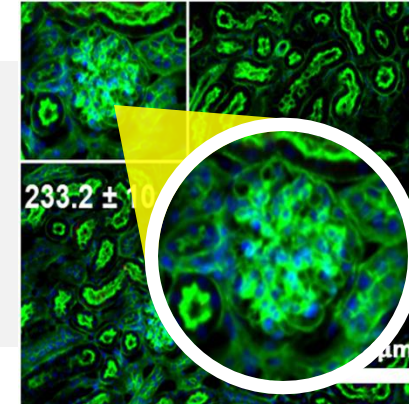
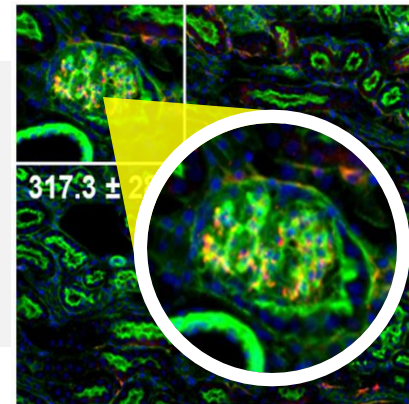
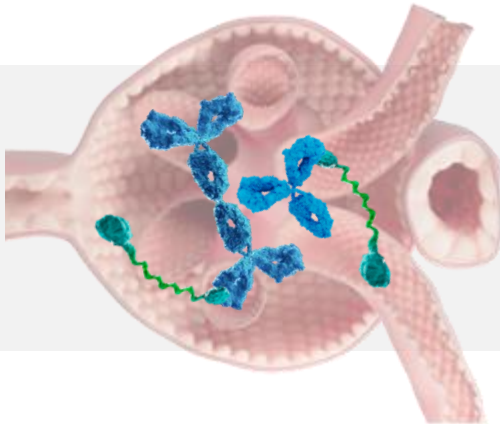
6 HOUR

Gd-IgA1  
+ Vehicle



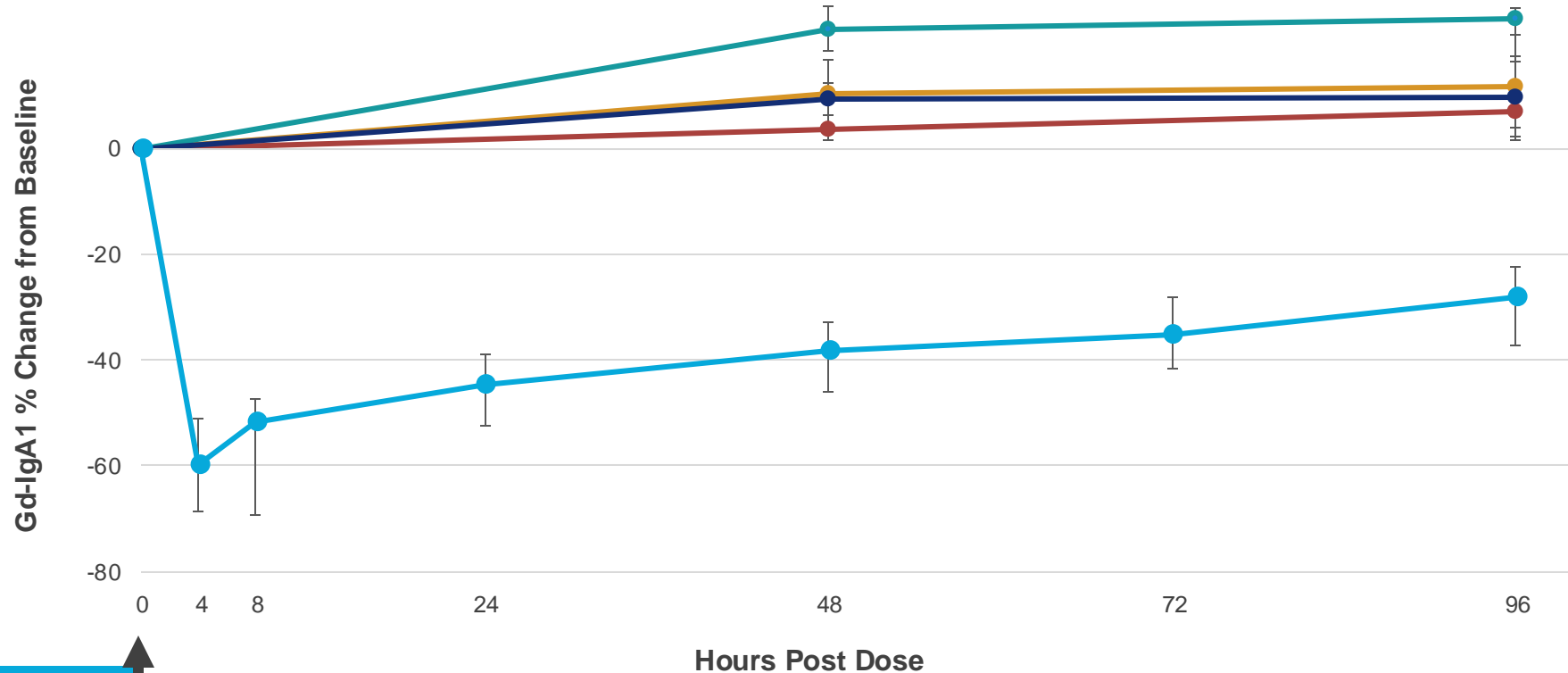
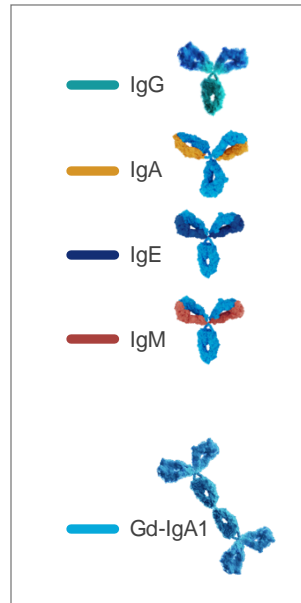
In the Absence of BHV-1400, Galactose-Deficient IgA1 (red) Accumulates in the Renal Glomerulus

Gd-IgA1  
+ BHV-1400



BHV-1400 Rapidly Clears Galactose-Deficient IgA1

# Preliminary Phase 1: Selective and Deep Removal of Gd-IgA1 Within Hours



**BHV-1400 125 mg  
Single dose**

**> 60% Gd-IgA1 LOWERING within 4 hours**

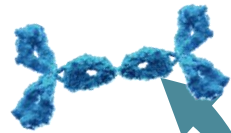


BHV-1400 at the lowest SAD cohort rapidly and selectively removes 60% of Gd-IgA1 while preserving normal immunoglobulins (IgG, IgE, IgA, IgM)

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

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

Gd-IgA1 autoantibody

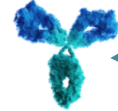


IgD



Tarpeyo  
**BROAD IMMUNOSUPPRESSION**  
 calliditas  
THE THERAPEUTICS

IgG

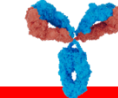


**B CELLS**

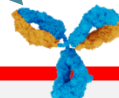
IgE



IgM



IgA



**TARGET B CELLS**

with global immunoglobulin suppression



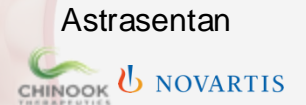
**INHIBITS COMPLEMENT SYSTEM**  
 With broad immunosuppression

Fabhalta

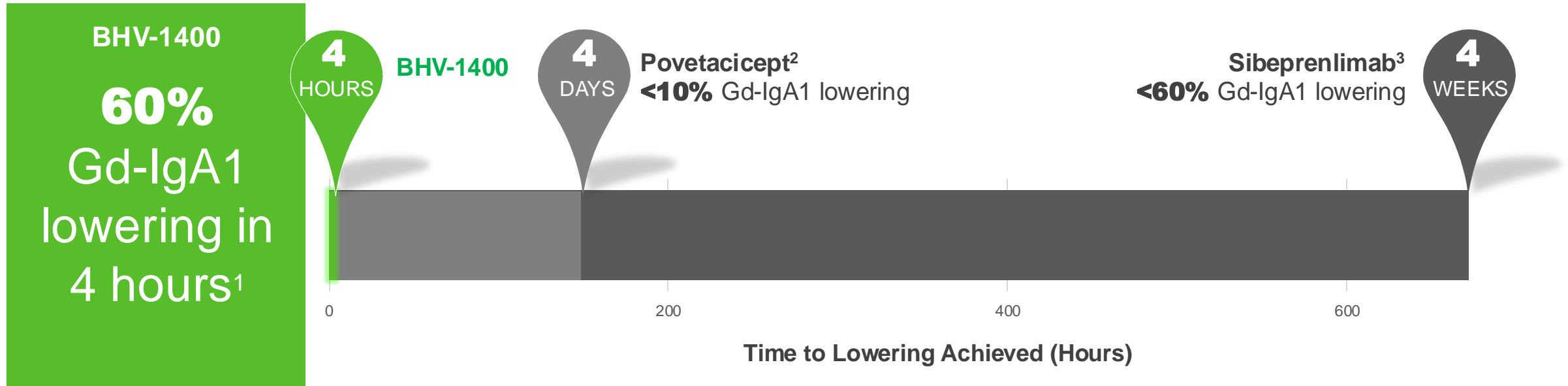
RO7434656

Ravulizumab (Ultomiris)

**TARGET ENDOTHELIN RECEPTOR**



# BHV-1400 Degrades Gd-IgA1 Rapidly: Timeline of Earliest Reported Gd-IgA1 Lowering Across Key Market Competition



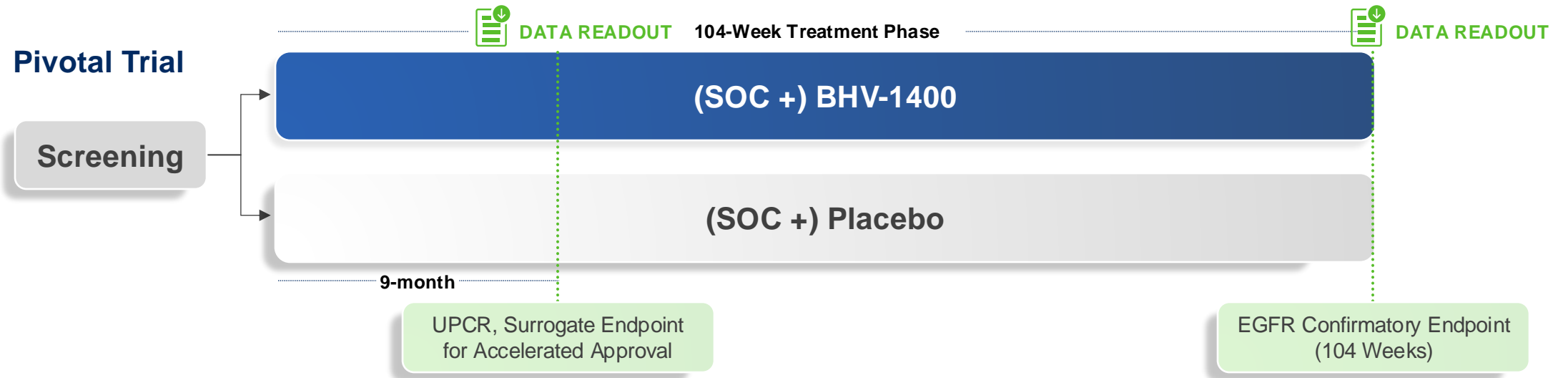
1. Lowering numbers reported for the median from the first and lowest BHV-1400 SAD cohort and for mean lowering for the highest dose SAD cohorts for Sibeprenlimab (12.0 mg/Kg) and Povetacicept (960 mg) 2. Davies et al. A first-in-human, randomized study of the safety, pharmacokinetics and pharmacodynamics of povetacicept, an enhanced dual BAFF/APRIL antagonist, in healthy adults. Clin Transl Sci. 2024 Nov;17(11):e70055. doi: 10.1111/cts.70055. PMID: 39494621; PMCID: PMC11532938. 3. Mathur et al. Safety, tolerability, pharmacokinetics, and pharmacodynamics of VIS649 (sibeprenlimab), an APRIL-neutralizing IgG2 monoclonal antibody, in healthy volunteers. Kidney Int Rep. 2022 Feb 8; 7(5): 993-1003. doi: 10.1016/j.ekir.2022.01.1073. PMID: 35570983; PMCID: PMC9091613.

**KEY  
POINT**

Lowest dose of BHV-1400 tested shows deep reductions of Gd-IgA1 within hours

# Harnessing Efficient Trial Design to Address a High Unmet Need

## BHV-1400 Phase 2/3 Study Concept



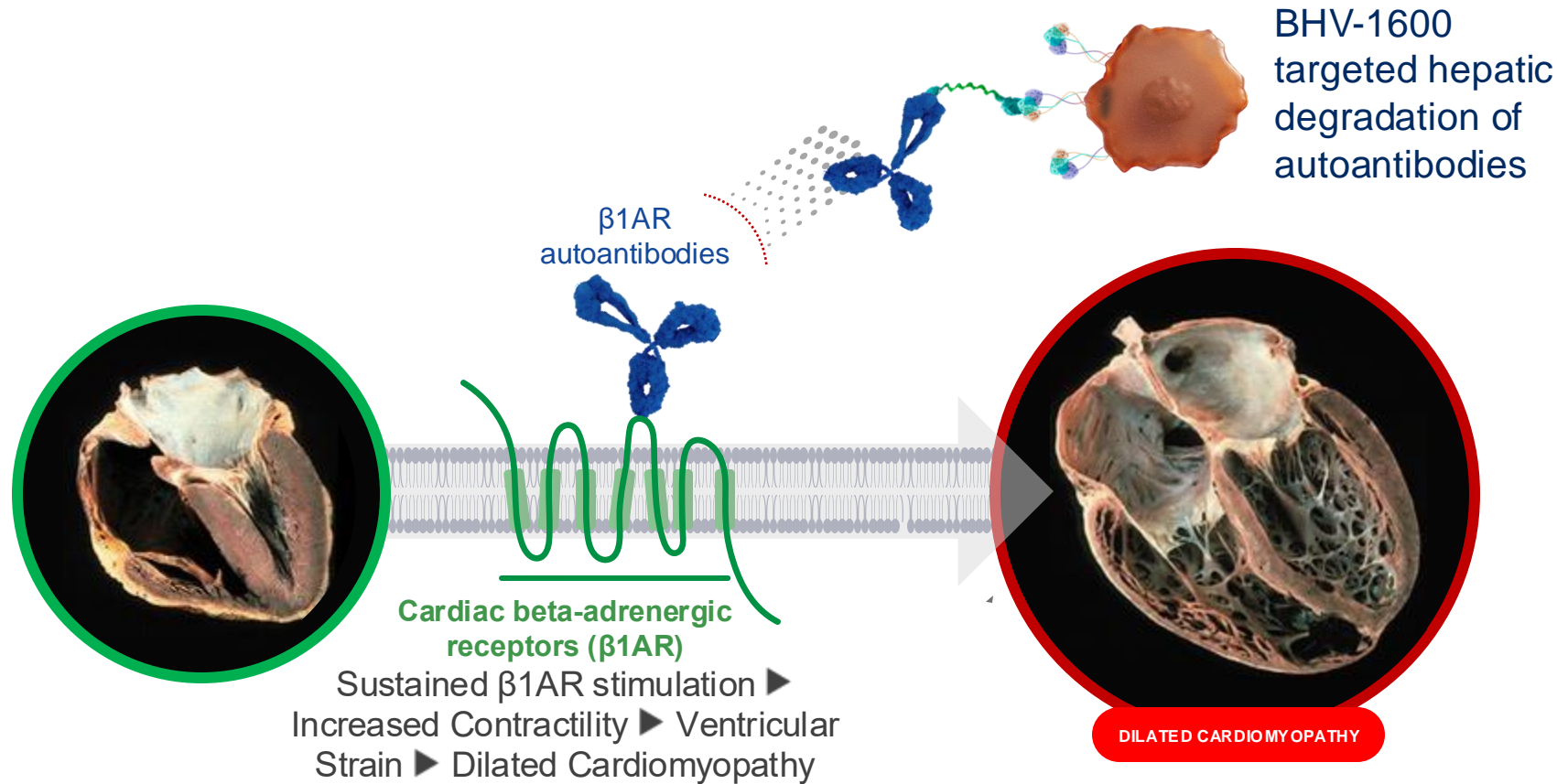


- ✓ BHV-1600: Potential to treat through selective removal of pathogenic autoantibody without chronic immunosuppression
- ✓ High unmet need: rare disease affecting new mothers with no approved treatment
- ✓ Robust science highlighting  $\beta$ 1AR-autoantibodies as pathogenic
- ✓ Biomarker endpoint with FDA-aligned path forward for accelerated approval

# PERIPARTUM CARDIOMYOPATHY



# BHV-1600, a Novel Investigational Treatment for Peripartum Cardiomyopathy



## PERIPARTUM CARDIOMYOPATHY:

- A rare disease with high unmet need
- 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)
- BHV-1600 degrades β1AR autoantibodies to potentially prevent irreversible heart failure

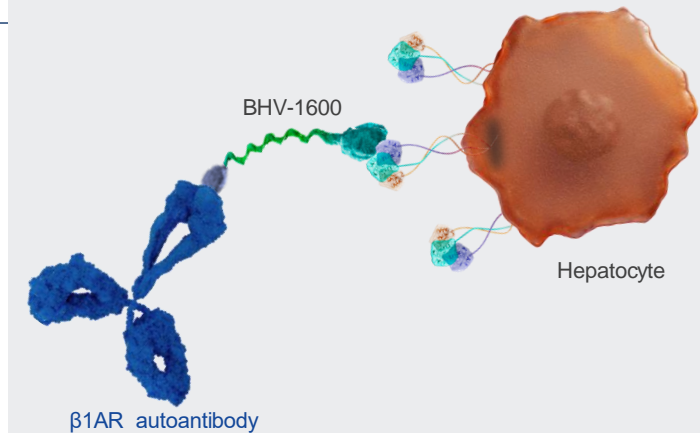
**KEY**  
POINT

BHV-1600 degrades β1AR autoantibodies to potentially prevent permanent heart failure in previously healthy mothers

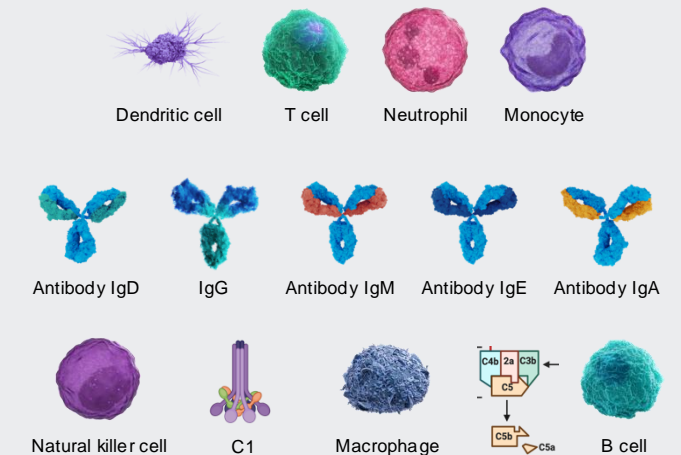
# Ongoing Phase 1 Preliminary Clinical Data

- **First-in-human dosing with BHV-1600 has been safe and well-tolerated to date with two cohorts dosed**
- **All AEs have been mild, with no SAEs**
- **Laboratory data demonstrate optimal safety profile:**
  - No clinically relevant changes in white blood cells or immunoglobulins IgG, IgA, IgE, and IgM
  - No clinically significant reductions in albumin, liver function test abnormalities, or increases in cholesterol compared to baseline
- **Study ongoing 1H 2025**

BHV-1600, designed to selectively degrade  $\beta$ 1AR autoantibodies to treat PPCM



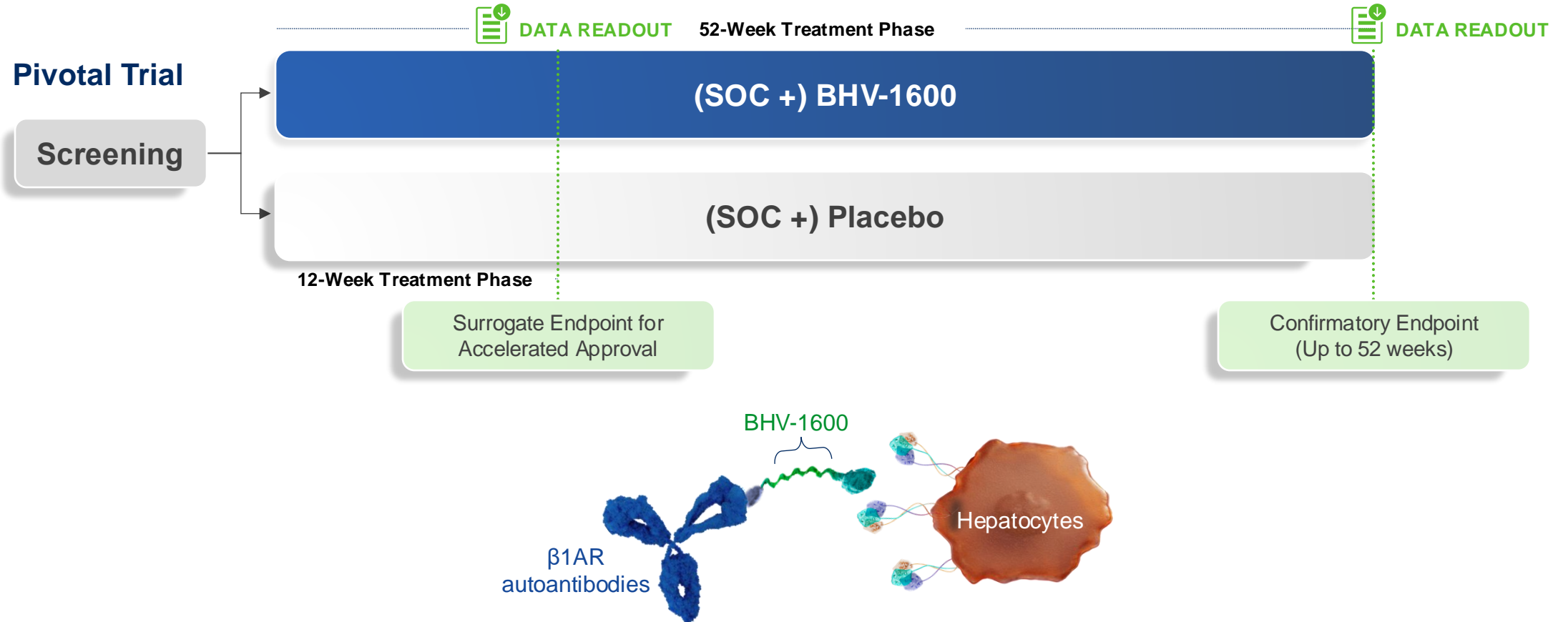
## While Preserving Immunity



**KEY**  
POINT

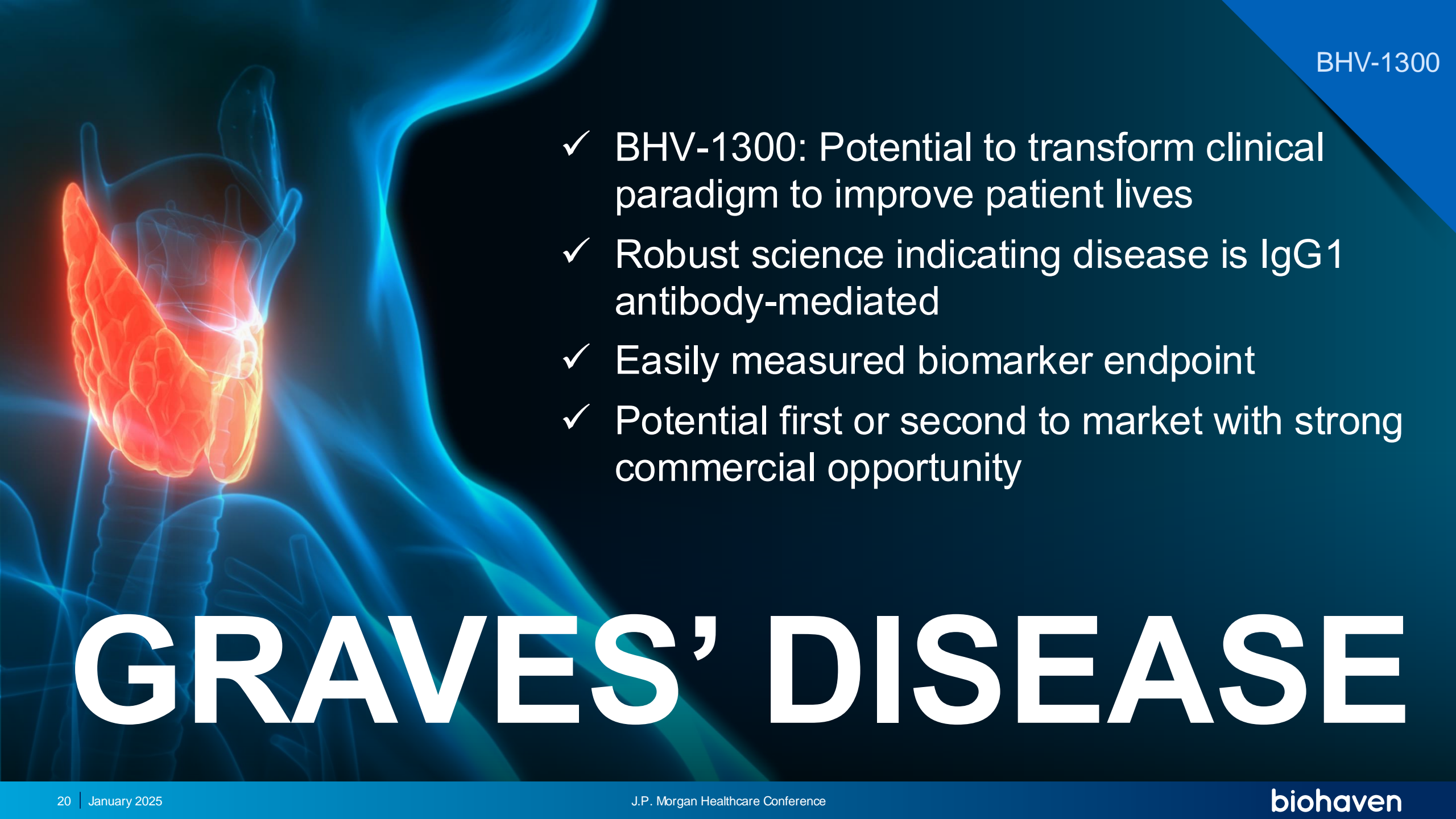
BHV-1600 selectively targets  $\beta$ 1AR autoantibodies to treat PPCM with Optimal Safety Profile

# Harnessing Efficient Trial Design to Address a High Unmet Need



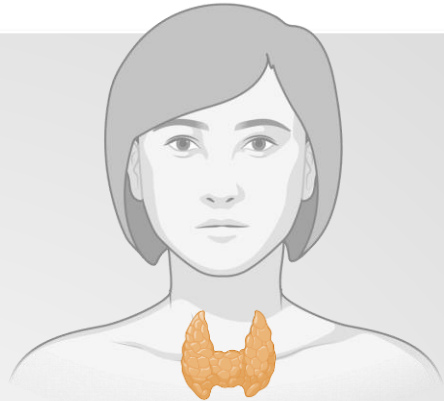
**KEY  
POINT**

Completed INTERACT meeting with FDA regarding accelerated approval pathway to bring a much-needed therapeutic to women with PPCM efficiently

- 
- ✓ BHV-1300: Potential to transform clinical paradigm to improve patient lives
  - ✓ Robust science indicating disease is IgG1 antibody-mediated
  - ✓ Easily measured biomarker endpoint
  - ✓ Potential first or second to market with strong commercial opportunity

# GRAVES' DISEASE

# Seventy Years of Research Demonstrate the Pathogenicity of TSH Receptor Autoantibodies in Graves' Disease (GD)



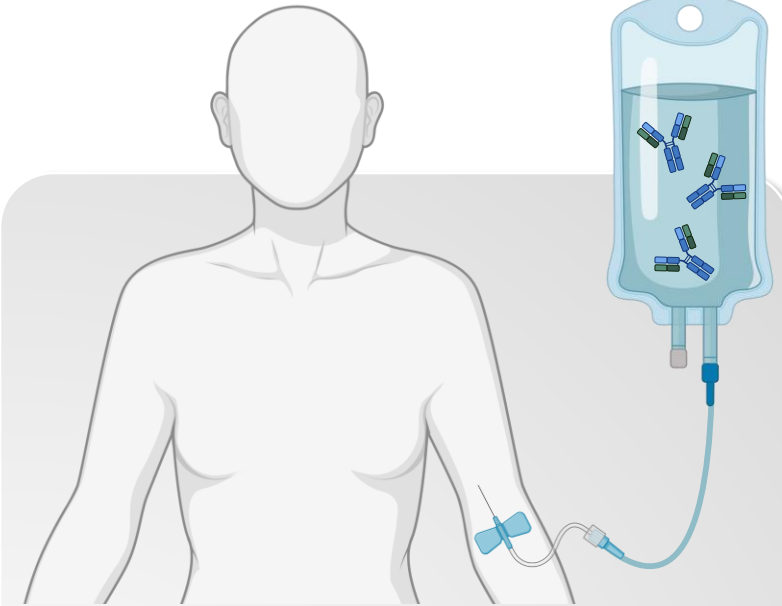
1950s

“Long-Acting Thyroid Stimulator,”  
(TSH receptor autoantibody)  
identified in the serum of GD patients



1960s

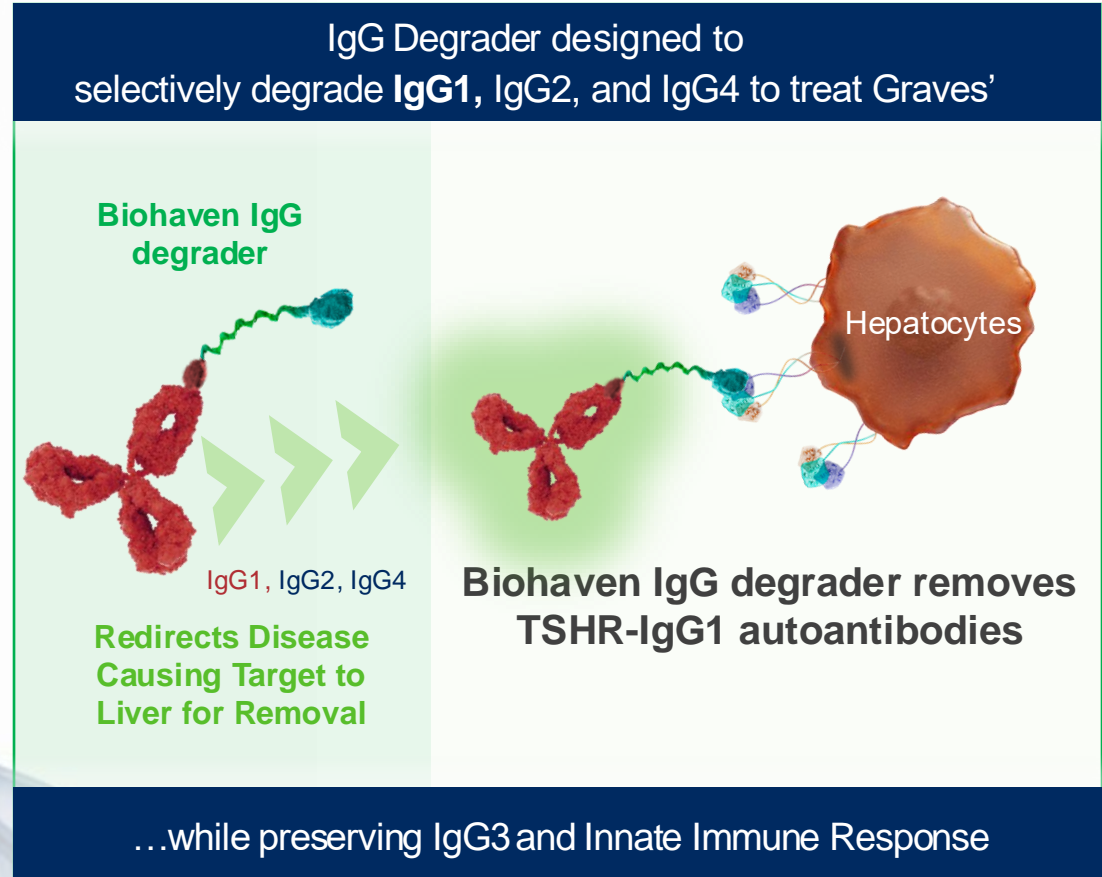
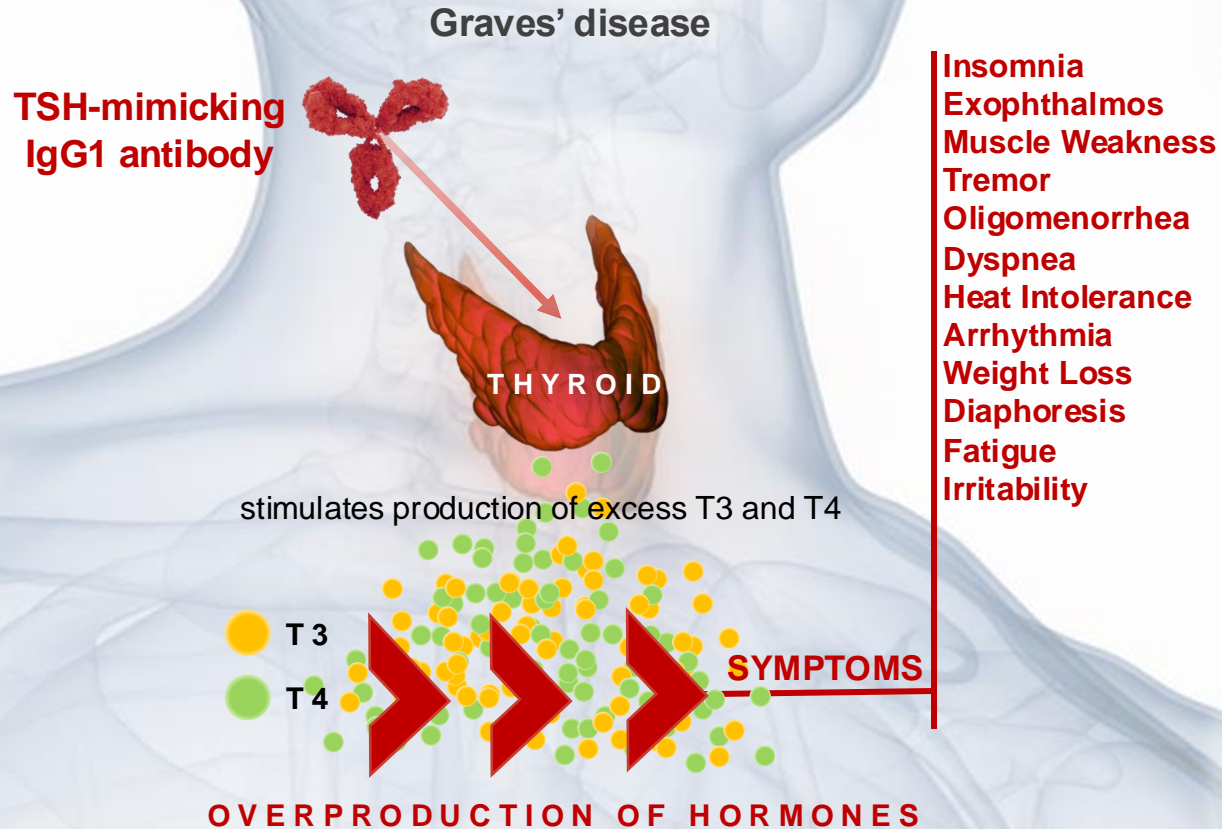
TSH receptor autoantibodies are  
detected in serum of neonates with  
hyperthyroid mothers with GD



1970s

Passive transfer of TSH receptor  
autoantibodies into healthies prove  
definitively to stimulate the thyroid

# Biohaven IgG1,2,4 Degradation Platform: A Novel Therapeutic for the Treatment of Graves' Disease



# Redefining Possibilities in Graves' Disease Treatment: Treat the Mechanism of Disease, Spare Patients their Thyroid



“Why lose my thyroid?”

“Why expose myself to radiation?”

“Why trade  
HYPERthyroidism for  
HYPOthyroidism?”

“A drug that causes fatal  
agranulocytosis and liver  
failure is probably not  
one I want to take.”

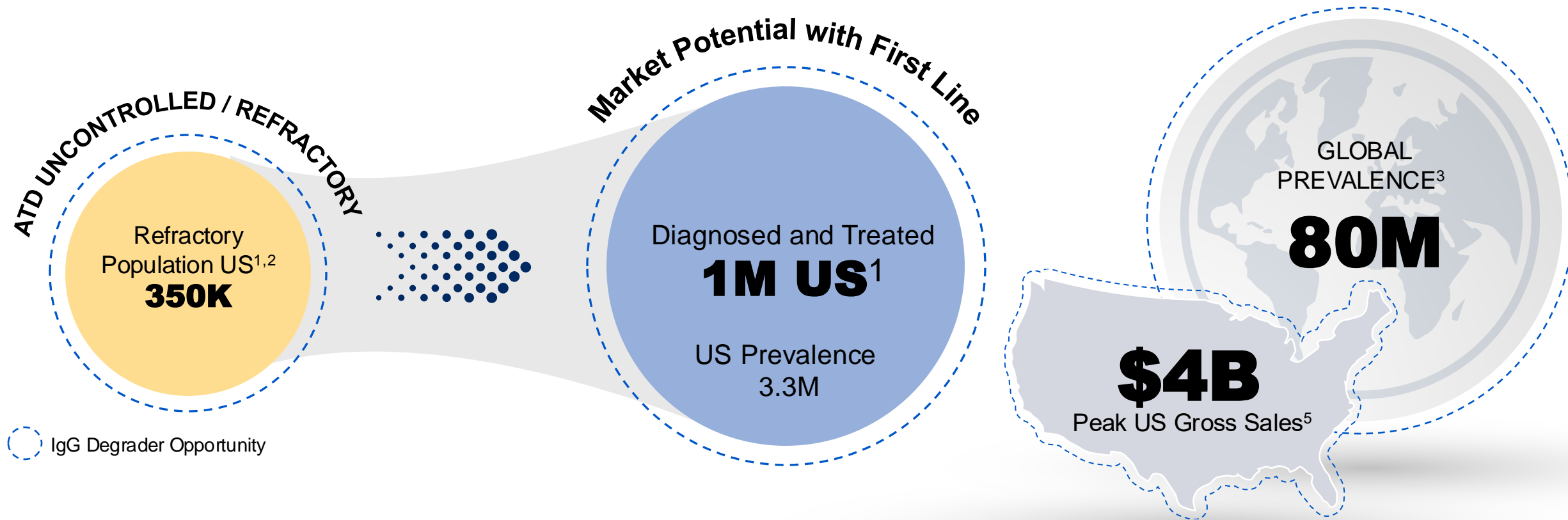
## LIMITATIONS OF ANTI-THYROID THERAPY (ATD)

- Does not treat the underlying autoimmune disease
- Are associated with birth defects
- Side effects include liver toxicity, agranulocytosis, hypothyroidism, allergic reactions, etc.
- Other treatment options like ablation or surgery invasive and causes permanent hypothyroidism resulting in life-long need for thyroid hormone replacement

**KEY**  
POINT

BHV-1300 targets the underlying autoimmune pathology of Graves' disease to potentially improve disease control and avoid the undesirable adverse effects of ATD's and surgery

# Broad Market Strategy to Modify Graves' Disease



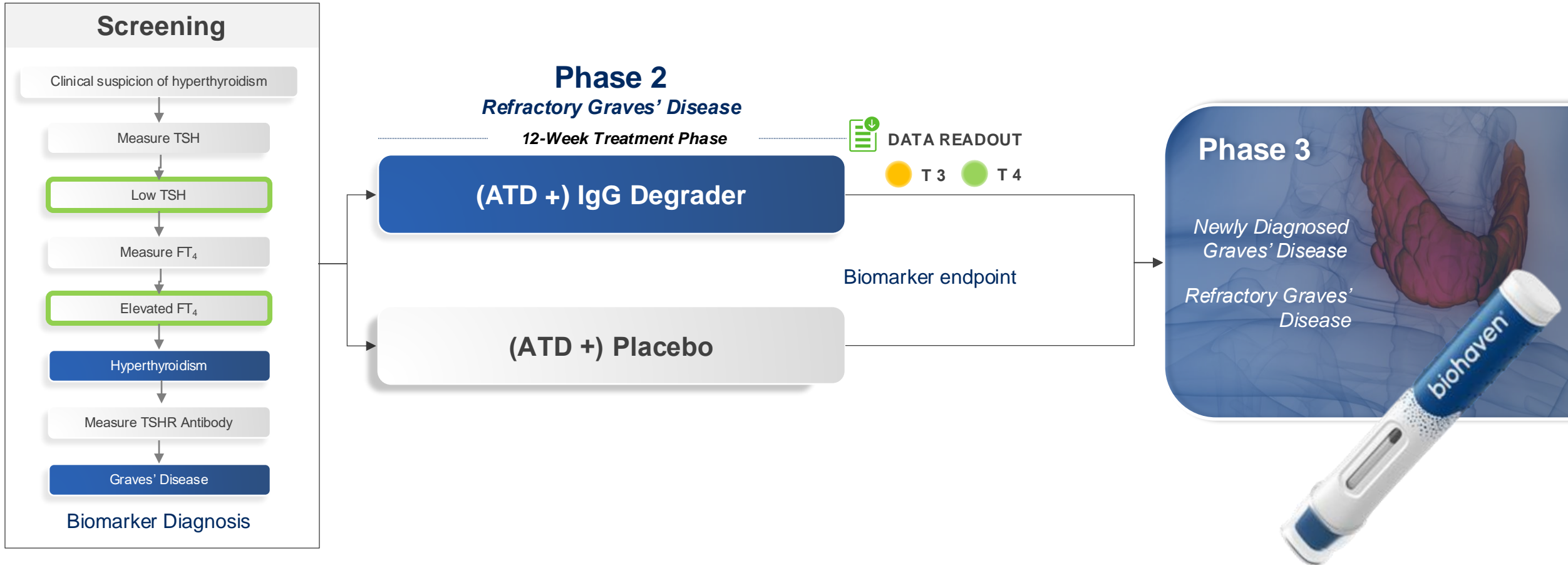
1. Forian Insurance Claims Data Base Analysis Jun 2016-September 2024; 2. Percent of ATD patients refractory or uncontrolled: Sundaresh V, Brito JP, Thapa P, Bahn RS, Stan MN. Comparative Effectiveness of Treatment Choices for Graves' Hyperthyroidism: A Historical Cohort Study. Thyroid. 2017 Apr;27(4):497-505. doi: 10.1089/thy.2016.0343. Epub 2017 Feb 6. PMID: 28049375; PMCID: PMC5385429; 3. NBK448195/NIDDKD. Graves disease. Accessed September 11, 2024. <https://www.niddk.nih.gov/health-information/endocrine-diseases/graves-disease>; 4. US prevalence and Incidence: Pokhrel B, Bhusal K. Graves Disease. [Updated 2023 Jun 20]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2024 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/>; 5. Biohaven Internal Analysis: Peak US Gross Sales

**KEY  
POINT**

Degraders redefine care, targeting the autoimmune pathogenesis of disease with the potential to treat across the course of disease



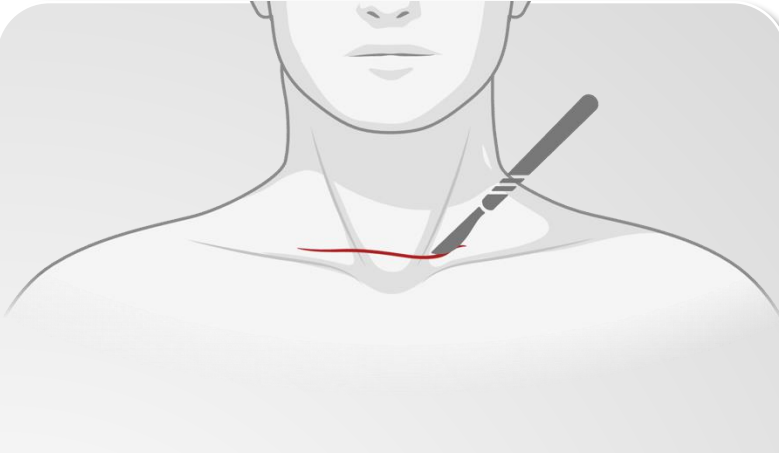
# Graves' Disease Mid-2025 with Biomarker Endpoint



**KEY  
POINT**

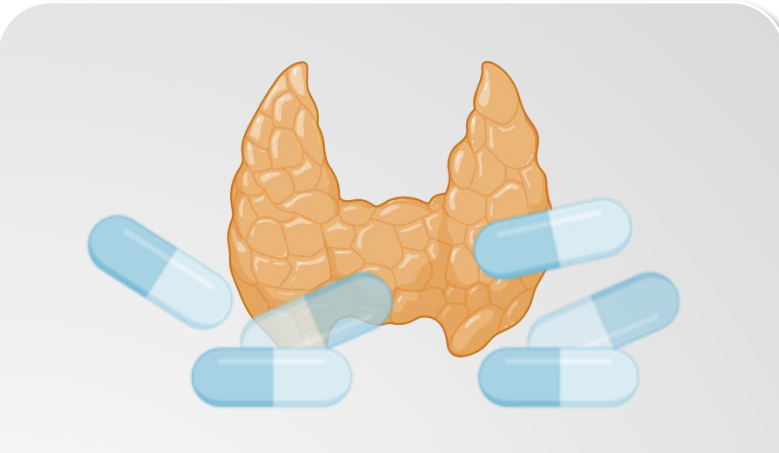
Biomarker-driven diagnosis and endpoints facilitate efficient trial design in Graves' disease patients

# Biohaven's Goal Is to Change the Treatment Paradigm in Graves' Disease



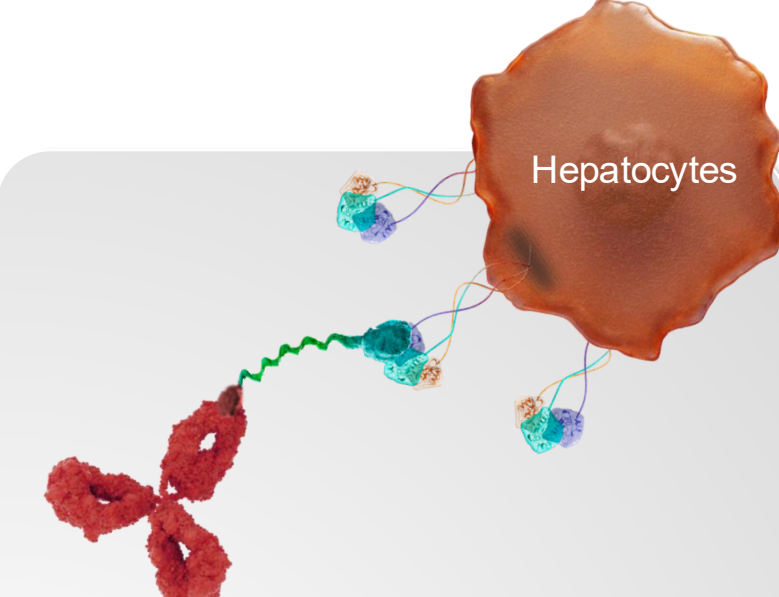
**1790s**

DeSault performs first successful partial thyroidectomy



**1940s**

Antithyroid drugs and radioactive iodine (RAI) used as alternative to surgery, chronic thyroid replacement



**2020s**

Biohaven technology redirects TSH receptor autoantibodies to the liver for removal, treating the underlying cause of Graves' disease

TRAb, TSH Receptor Autoantibodies

# Market Potential of Biohaven's Degrader Platform

> \$15B\*

~ \$8B\*

**IgG DEGRADERS**  
 Graves' disease  
 Rheumatoid arthritis (RA)  
 Myasthenia gravis (MG)

**β1AR AUTOANTIBODY DEGRADER**  
 Dilated cardiomyopathy (DCM)  
 Peripartum cardiomyopathy (PPCM)

**Gd-IgA1 DEGRADER**  
 IgA Nephropathy  
 IgA Vasculitis

**IgG DEGRADERS**

**PROINSULIN AUTOANTIBODY DEGRADER**

**IgG4 DEGRADER**

**β1AR AUTOANTIBODY DEGRADER**

**Gd-IgA1 DEGRADER**

**IgG DEGRADERS**

**FUTURE DEGRADERS AND INDICATIONS**

**TSHR AUTOANTIBODY DEGRADER**

**PLA2R AUTOANTIBODY DEGRADER**

**PROINSULIN AUTOANTIBODY DEGRADER**

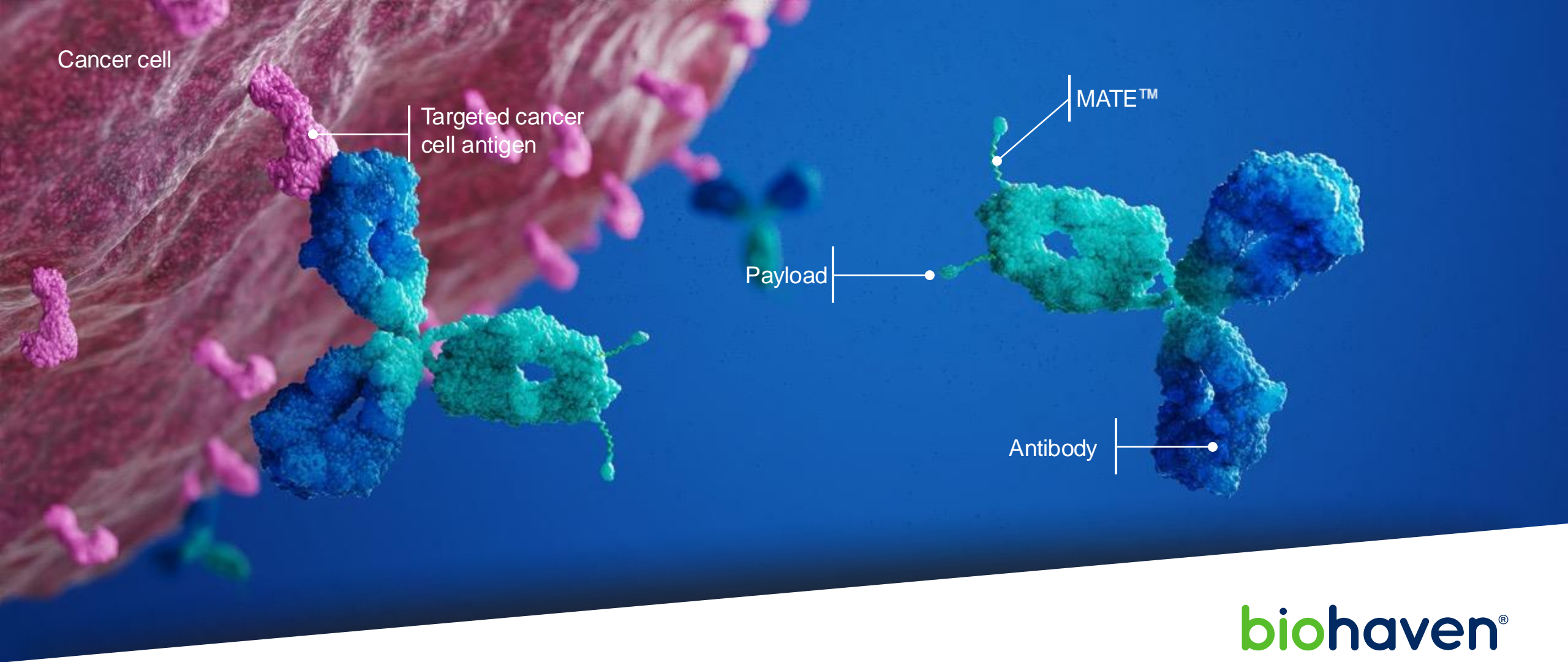
**IgG4 DEGRADER**

**β1AR AUTOANTIBODY DEGRADER**

**Gd-IgA1 DEGRADER**

**IgG DEGRADERS**

\* Biohaven Internal Analysis: Peak US Gross Sales



Cancer cell

Targeted cancer cell antigen

MATE™

Payload

Antibody

biohaven®

# Oncology: Next-Generation ADCs

# Biohaven's Novel ADC Conjugation Technology and Strategic Collaborations Driving Next-Generation Cancer Therapies

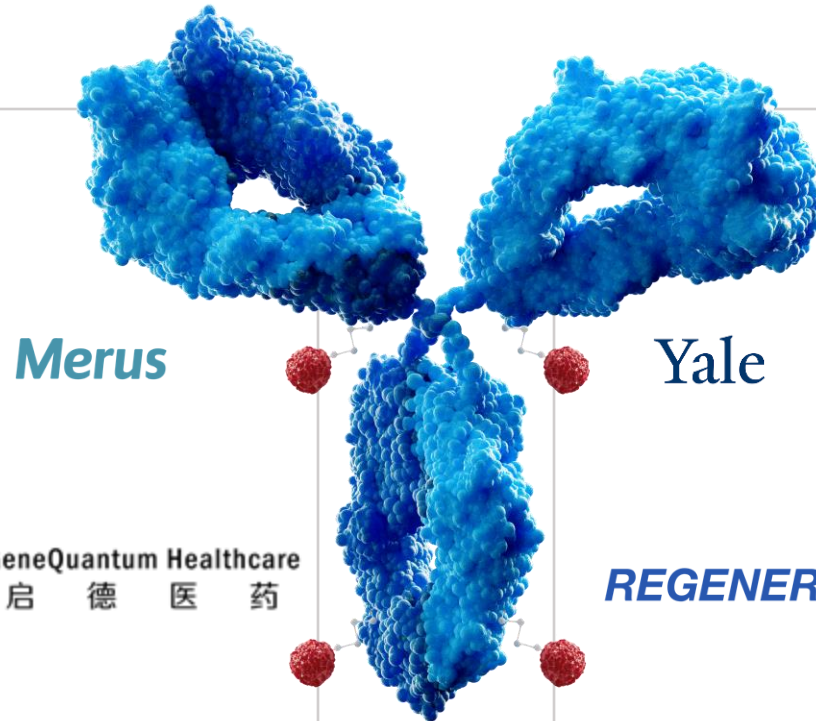
Collaborate to generate highly differentiated ADCs

## Novel mAbs

- Validated and emerging targets
- Merus collaboration leverages differentiated dual-targeting antibody platform

## Exclusivity to Topolx payload

- Superior preclinical anti-PD/L1 synergy and immunogenic cell death
- GeneQuantum collaboration provides broad target exclusivity to the payload for 18 oncology targets



Broad and flexible platform applicability

## Single-step chemistry, native mAbs

Modular, efficient, and scalable MATE<sup>®</sup> technology developed from Yale University Spiegel Lab

## Irreversible, Site-Specific Conjugation

Minimize payload-associated tox, DAR homogeneity

## Combination I/O Therapies

Supply agreement: BHV-1510 with Libtayo<sup>®</sup>

**BHV-1510** (Trop2 Topolx) in Phase 1 (mono and anti-PD1 combination)

**BHV-1530** (FGFR3 Topolx) in Phase 1 startup — FPI early 2025

**BHV-1500** (CD30 MMAE) IND planned 2025

Novel ADCs and De-Risked Fast-Followers in Clinic

**STRATEGIC COLLABORATIONS AND CLINICAL SUPPLY AGREEMENTS**

**MULTIPLE DC/INDs** planned 2025–2026

## BHV-1510 is a Highly Differentiated Trop2 ADC

- Ideally positioned for fast-to-market strategy with anti-PD-1 combo

## Novel Topolx Payload Synergy with Anti-PD-1 *In Vivo*

- Induces immunogenic cell death and complete tumor regressions
- Superior to datopotamab deruxtecan (DS-1062) plus anti-PD-1

## Fully Optimized Next-generation ADC

- Novel and highly stable linker-payload (DAR4)

## Differentiated Pre-clinical Safety Profile

- Datopotamab deruxtecan (DS-1062): interstitial lung disease (ILD)
- Sacituzumab tirumotecan (MK2870/SKB264): hematological toxicities
- TRODELVY®: neutropenia, diarrhea

## Milestones Achieved

- First-in-human trial initiated April 2024
- Anti-PD-1 combo cohorts with Libtayo® initiated 4Q 2024



**BHV-1510**  
TROP2 ADC

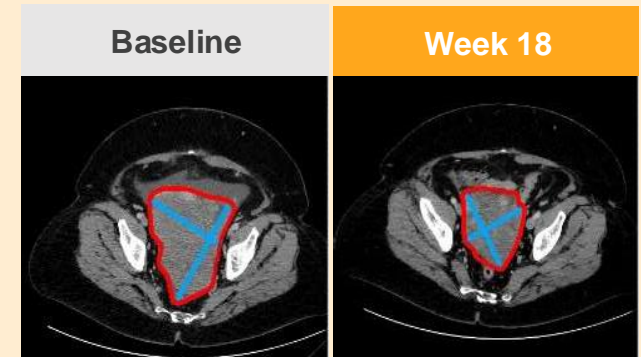
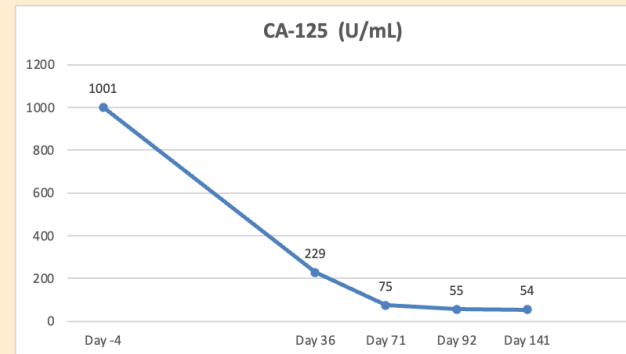
**BREAKING  
NEWS**

- Clinical activity and no ILD with Topolx observed in early cohorts
- Target exclusivity expanded for up to 18 ADC targets incorporating Topolx payload

# BHV-1510 (Trop2 ADC with Topolx) with Early Clinical Activity in Phase 1

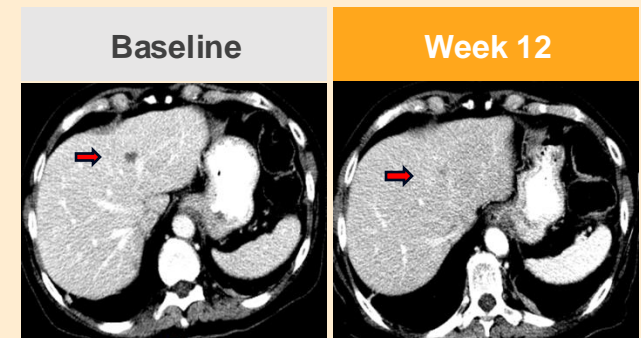
- Clinical activity across doses starting at the lowest dose (2 mg/kg, Q3W)
  - Tumor reduction observed in tumor types including ovarian, SCLC, NSCLC
- Favorable preliminary safety and PK profile
  - No payload-associated ILD, diarrhea, or significant hematological toxicity
  - Main toxicity observed is on-target Trop2 ADC class mucositis; an expected and manageable effect
  - Very low free payload in serum, demonstrates high ADC stability
- Dose escalation (mono and Libtayo<sup>®</sup> combo) and dose/schedule optimization ongoing

**Case 1:** 71 y/o, Platinum-resistant ovarian cancer, 2 mg/kg, Q3W  
25% tumor reduction at week 18 with dramatic drop in CA-125



**Case 2:** 70 y/o, SCLC post carboplatin+durvalumab and lurbinectedin, 4 mg/kg Q3W

PR (~60% reduction) at week 12



**KEY  
POINT**

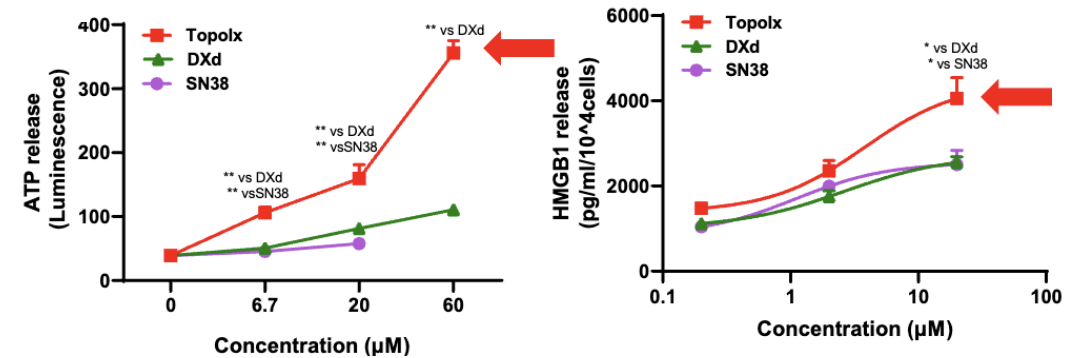
Observed clinical activity and safety supports broad investigation of ADCs incorporating novel Topolx payload and highly stable linker

# Topolx Payload Is a Novel Topoisomerase 1 Inhibitor With a Superior Pre-clinical Profile Compared to DXd and SN-38

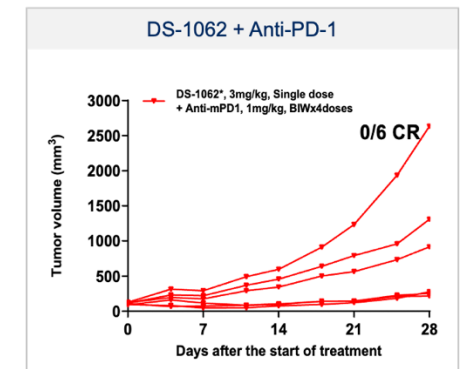
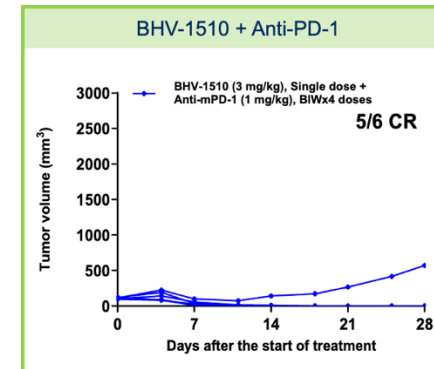
## Superior Pre-clinical Profile

	SN-38	DXd	Topolx
<i>In vitro</i> cytotoxicity	++	++	+++
ICD*	+	+	++
Transported by ABCG2	n/a	Y	N
Bystander killing	n/a	++	+++
<i>In vivo</i> efficacy	+	++	+++

## Superior Immunogenic Cell Death



## Synergy with anti-PD1 Combination





## Advancing Topolx Payload in Next-Gen ADC to Target Urothelial Cancer and Other Solid Tumors

- Novel and proprietary FGFR3 mAb
- Enzymatic, site-specific conjugation
- Favorable nonclinical tox profile

### Validated target with limited competition

- No ADCs approved or in advanced development
- Core opportunity in FGFR3-altered metastatic urothelial cancer (mUC)
  - only 1 Tyrosine Kinase Inhibitor approved
- Potential extension into other FGFR3-driven solid tumors
- ~\$400M to > ~\$1B peak US gross sales potential

### Synergistic Efficacy With Checkpoint Inhibitors *In Vivo*

- BHV-1530/anti-PDL1 combination showed synergy similar to BHV-1510
- PD1 synergy with PADCEV<sup>®</sup> (Nectin-4 ADC with MMAE payload) showed dramatically improved survival in mUC

### Milestones Achieved

- US FDA IND May Proceed Letter granted



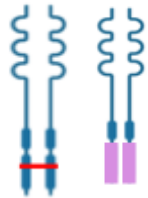
**BHV-1530**  
CLINIC-READY FGFR3 ADC

BREAKING  
**NEWS**

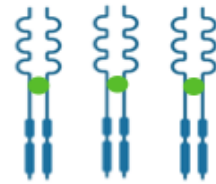
First-in-Human study planned to initiate in 1H 2025

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

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



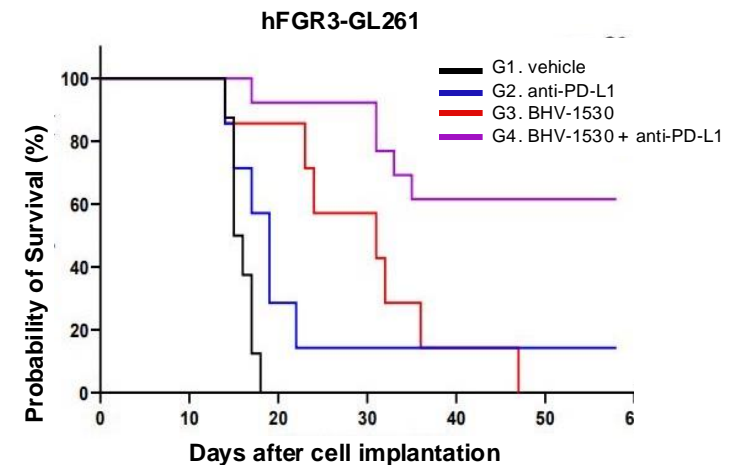
**FGFR3**  
mutation/fusion in  
~20% mUC



**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

# Biohaven-Merus Collaboration Represents a Leading-Edge Approach to Developing Highly Optimized Bispecific ADCs

## Merus

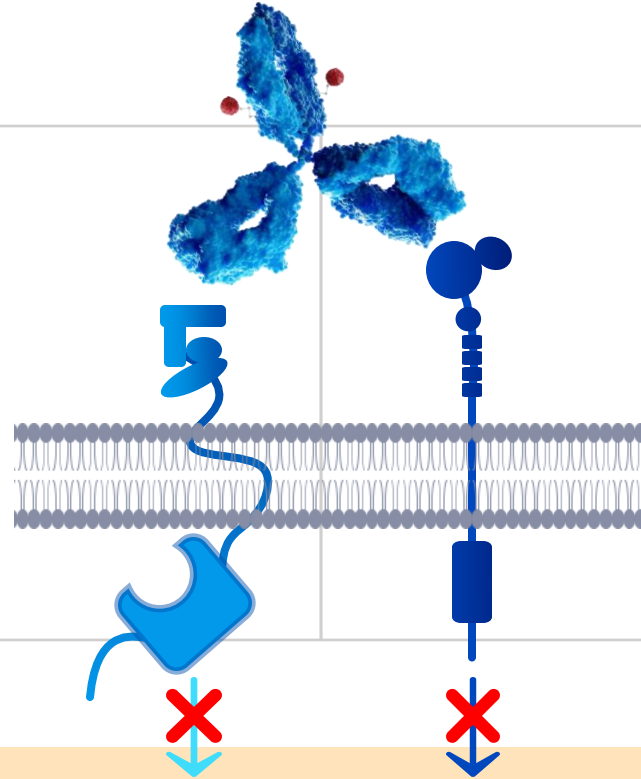
### A leader in developing differentiated bispecific mAbs for oncology

- Clinically validated platform
- Lead program (Zenocutuzumab) granted US FDA accelerated approval in December 2024

## biohaven

### Next-generation ADC conjugation and payload platform technologies

- Potential for superior specificity and benefit/risk profile vs. single target ADCs
- Co-development maximizes expertise and efficiencies

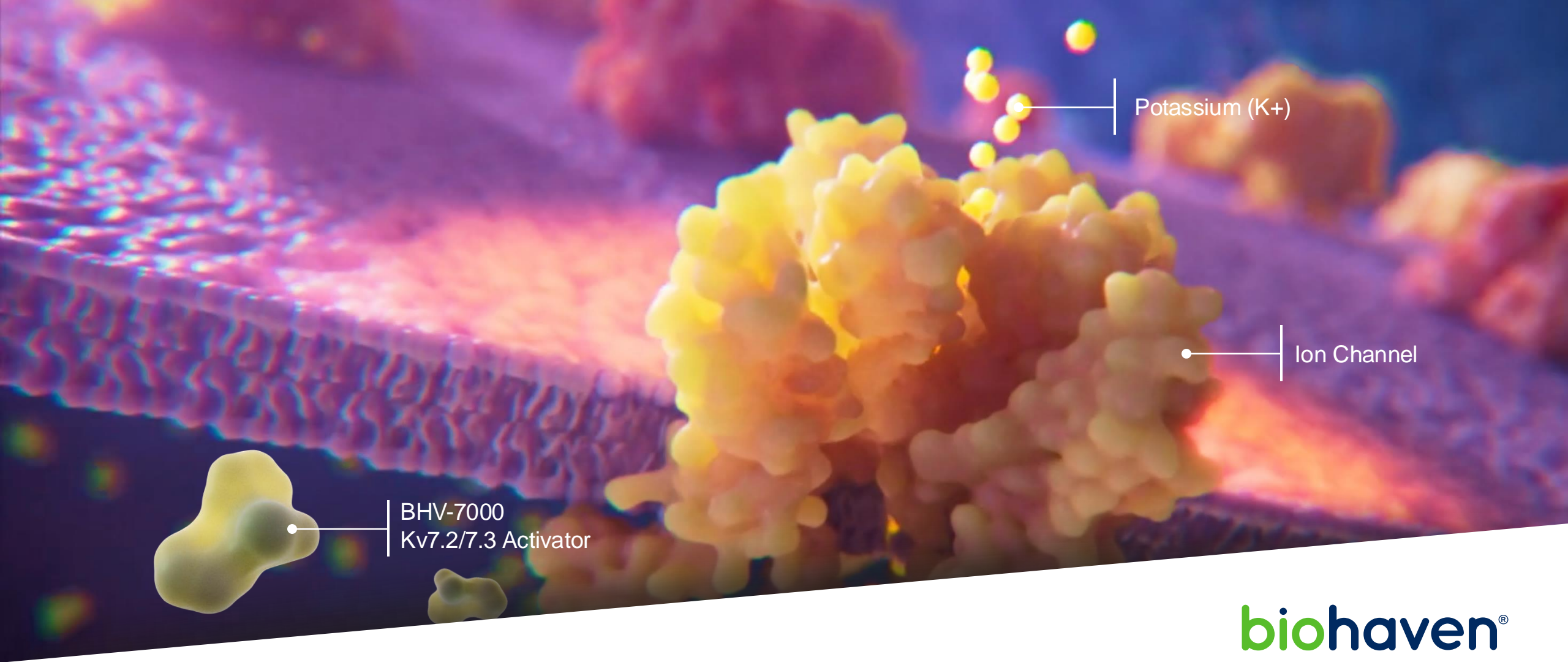


### Potential advantages of dual-target bispecific ADCs

- Preferential binding
- Improved internalization
- Optimal tumor penetration
- Multiple MOA of tumor cell killing

**BREAKING NEWS**

Multi-target collaboration, leveraging each company's innovative tech for ADC co-development



biohaven®

## Ion Channel Platforms

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



## Bipolar Disorder 7M Patients

- Novel MOA for bipolar disorder
- Differentiated profile vs. antipsychotics, lithium, and ASMs

Acute bipolar mania topline results expected in 1H 2025



## Major Depressive Disorder 21M Patients

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

Topline results expected in 2H 2025



## Epilepsy 3.5M Patients

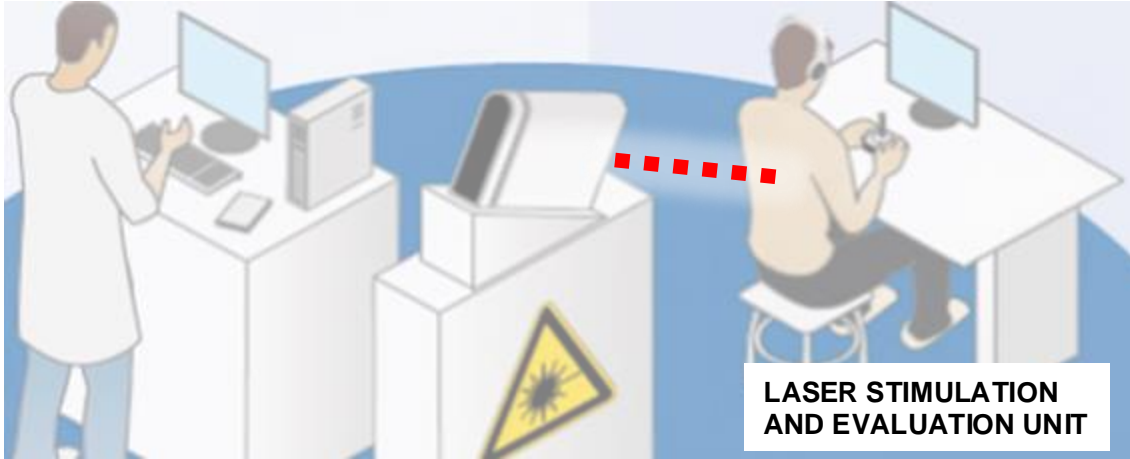
- Clinically validated MOA for epilepsy
- Global Phase 2/3 program ongoing in focal epilepsy (2 trials) and idiopathic generalized epilepsy (1 trial)

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

**BREAKING  
NEWS**

Pivotal topline results for BHV-7000 development program expected within the next year

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

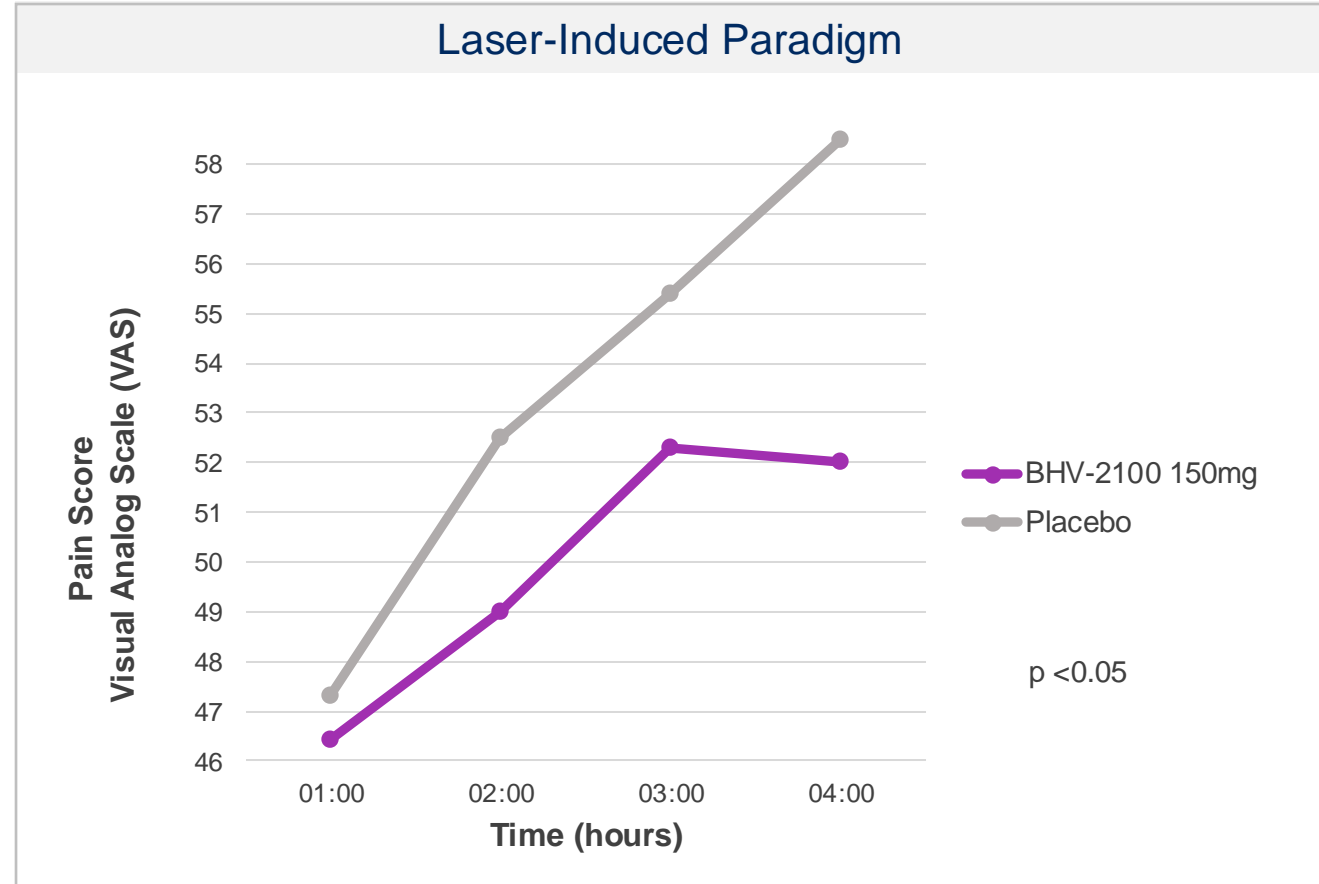


## Efficacy

- 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

POSTSYNAPTIC NEURON

TRORILUZOLE

GLUTAMATE

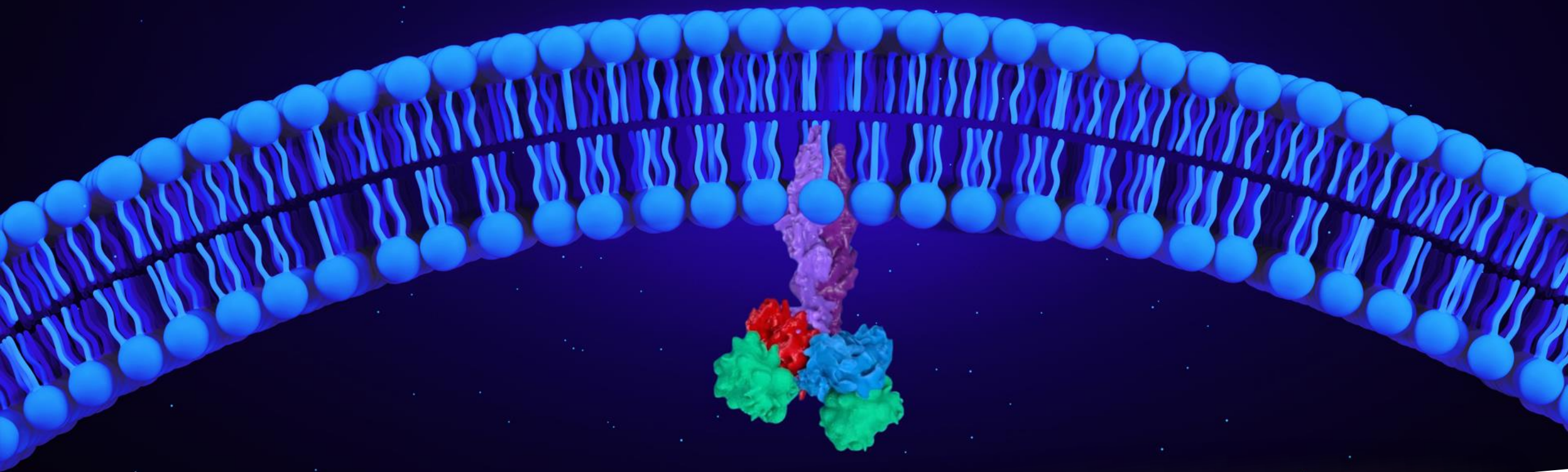
PRESYNAPTIC NEURON

biohaven®

Troriluzole — SCA







BHV-8000

biohaven<sup>®</sup>

## First-in-Clinic, Oral, Selective, Brain-Penetrant TYK2/JAK1 Inhibitor

- Uniquely potent, TYK2/JAK1 selective, brain-penetrant inhibitor
- Selectivity profile avoids class risks associated with JAK2/3 inhibition

## Breaks the Cycle of Neuroinflammation

- Reduces inflammatory impacts of microglia, astrocytes and infiltrating T-lymphocytes

## Potential to Treat Multiple Neuroinflammatory Disorders

- Supported by a broad range of clinical, translational, and epidemiological evidence
- Indications include Parkinson's disease, anti-amyloid therapy induced ARIA, Alzheimer's disease, and multiple sclerosis

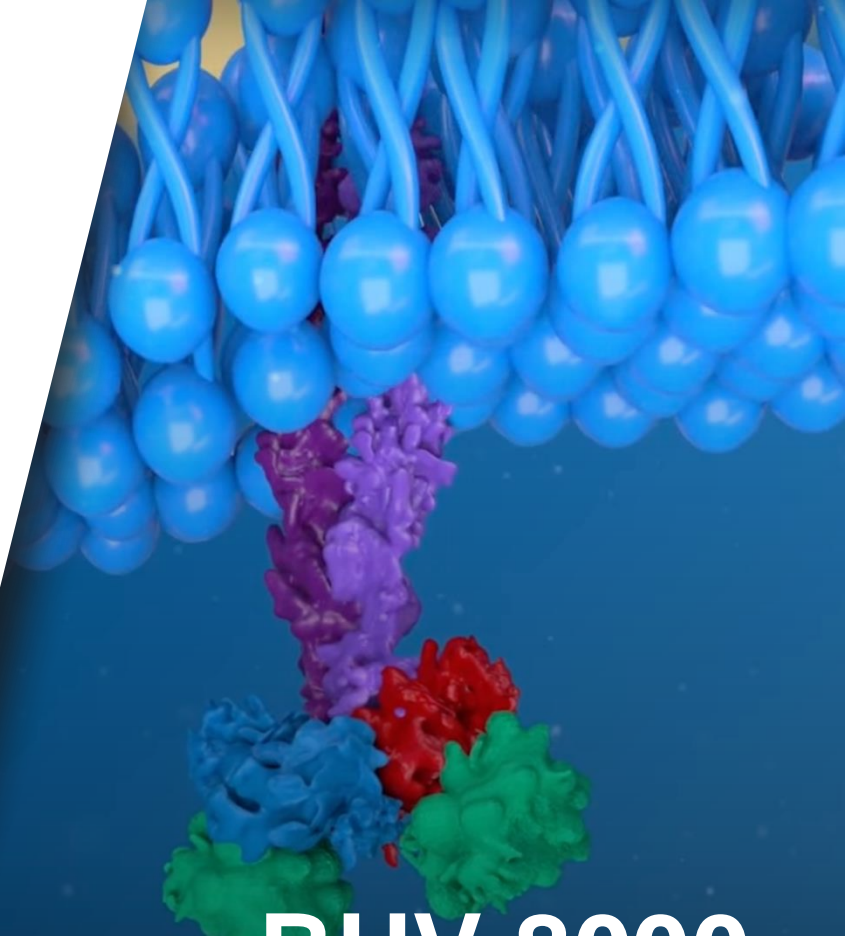
## Encouraging Results from Completed Phase 1 Trial

- Safe and well-tolerated
- Evidence of target engagement
- Robust brain penetration

## Milestone Achieved

FDA meetings successfully completed enabling registrational programs for Parkinson's disease and prevention of ARIA

ARIA, Amyloid-related imaging abnormalities; SAD, single ascending dose; MAD, multiple ascending dose; TYK, tyrosine kinase; JAK, Janus kinase.



**BHV-8000**  
**TYK2/JAK1 INHIBITOR**  
(brain-penetrant)

**BREAKING  
NEWS**

Pivotal study in Parkinson's disease planned to initiate in 1H 2025

# 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	<b>0.66</b>	0.77 (0.74 – 0.80)	<0.0001
No Treatment	50,562	5,328,307	0.95		
Anti-TNF exposure	2,471	371,867	<b>0.66</b>	0.64 (0.52 – 0.80)	<0.0001
No Treatment	50,562	5,328,307	0.95		
Anti-IL-17 exposure	81	15,598	<b>0.52</b>	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.

# BHV-8000 Demonstrates a Promising Phase 1 Profile

## **STUDY COMPLETED: 3 SAD cohorts and 3 MAD cohorts**

- SAD dose cohorts (10, 20 and 30 mg); MAD dose cohorts (6, 10 and 20 mg)
- 8 healthy subjects per cohort (6 active: 2 placebo)

## **SAFETY PROFILE: Safe and well-tolerated to date**

- No SAEs or severe AEs; only mild AEs observed that resolved spontaneously
- No adverse laboratory trends related to study drug

## **PHARMACODYNAMIC EFFECTS**

hs-CRP, IFN-beta, and IP-10 showed drug-related changes in plasma

## **PHARMACOKINETICS**

Approximately 50% CNS penetration in humans

AE, adverse event; hs-CRP, high-sensitivity C-reactive protein; IFN-beta, Interferon beta; MAD, multiple ascending dose; SAD, single ascending dose; SAE, serious adverse event.

**KEY**  
POINT

BHV-8000 is safe and well-tolerated at doses showing evidence of CSF penetration and target engagement

# BHV-8000: Unique Phase 2/3 Study Design for Parkinson's Disease

## Novel Primary Efficacy Endpoint

### Time-to-event ( $\geq 2$ -point worsening on MDS-UPDRS-Part II)

- Addresses FDA requirement for a functional endpoint in PD trials
  - MDS-UPDRS-Part II recommended, but declines very slowly in early PD
- 300 fewer patients per trial versus a mean change on MDS-UPDRS-Part II
- Based on comprehensive analyses of PPMI and placebo-arm clinical trial data (C-Path)

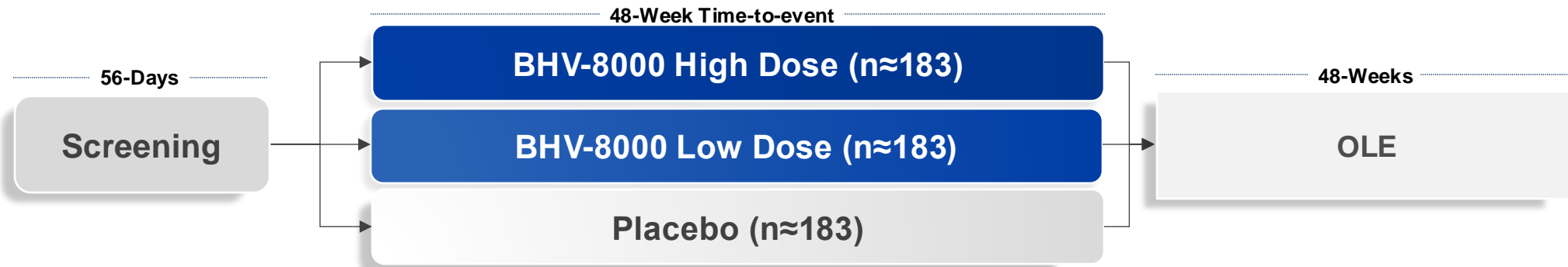
Provides a meaningful efficacy endpoint with a smaller sample size

## Novel Composite Endpoint

### Parkinson's Disease Composite Score (PARCOMS)

- Based on established methodology (i.e., Alzheimer's Disease Composite Score [ADCOMS])
- Leverages PPMI and placebo-arm clinical trial data (C-Path)
- Comprises the most responsive items from common endpoints in early PD trials

Provides a highly-sensitive supportive secondary efficacy endpoint



PPMI, Parkinson's Progression Markers Initiative; MDS-UPDRS, Movement Disorder Society – Unified Parkinson's Disease Rating Scale.

**BREAKING  
NEWS**

Pivotal study planned to initiate in 1H 2025

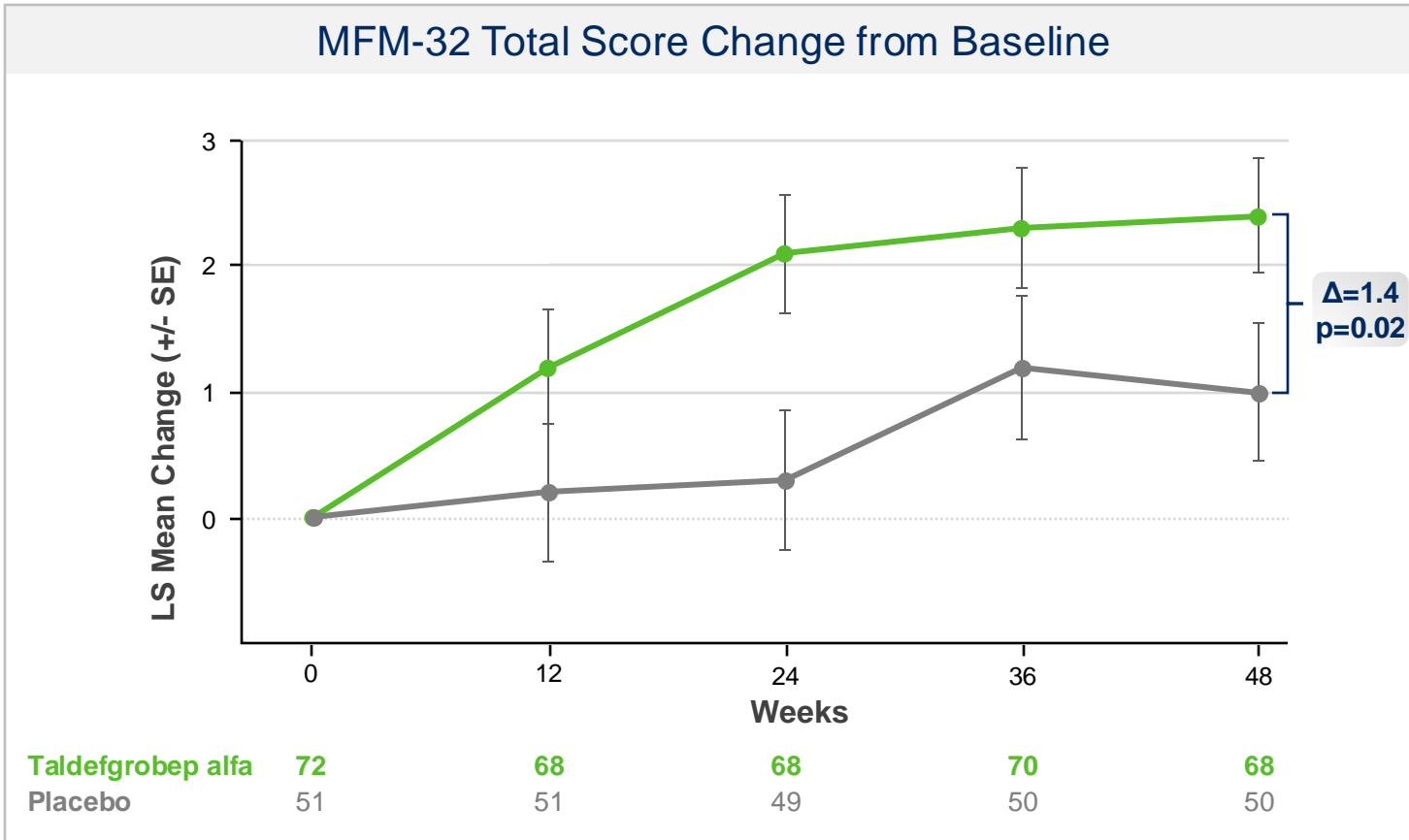
SKELETAL MUSCLE CELL SURFACE



biohaven®

Myostatin — SMA and Obesity

# Efficacy Results: Clinically Meaningful Improvements Enhanced In Myostatin-Positive Caucasian Participants



## ADDITIONAL SUPPORTIVE DATA

- *Responder Analysis\**  
50% of taldefgrobep-treated participants responded vs. 30% on placebo
- *Open-label Extension\*\**  
Motor function continues to improve

## Taldefgrobep Significantly Reduced Fat Mass Gain in SMA Participants While Increasing Lean Muscle Mass and Bone Density (vs. Placebo)

DXA prespecified outcome measures in overall study population at Week 48 demonstrated:

- Greater reduction in percent change in total body **fat mass** (p=0.008)
- Numerically larger increases in **lean muscle mass**
- Numerically larger increases in **bone density**

LS, least squares; MFM-32, 32-Item Motor Function Measure; SE, standard error

\* response defined as ≥ 3-point change from baseline improvement on MFM-32 at Week 48 \*\*Preliminary data

**KEY  
POINT**

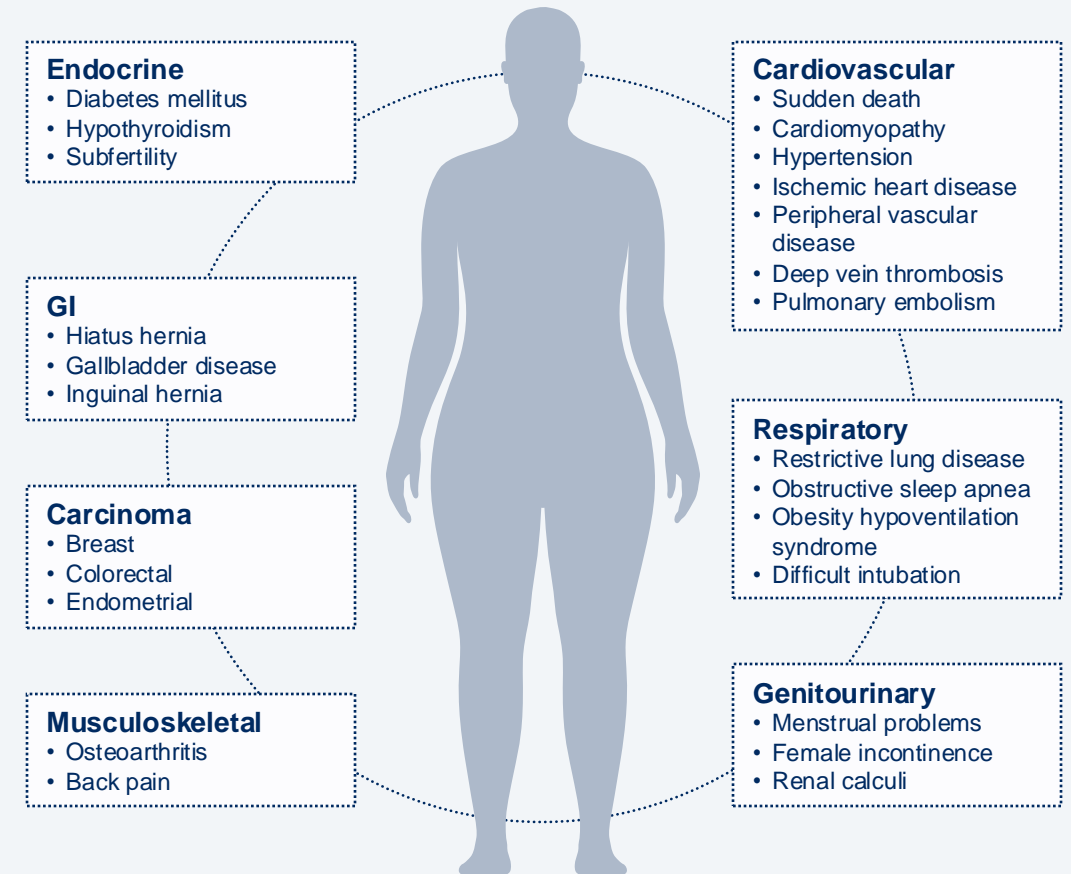
Placebo adjusted difference similar to what was seen with other SMA therapy (risdiplam) in registrational SUNFISH trial; magnitude of effect appears additive since added to SOC

# 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<sup>1</sup>
- 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<sup>2</sup>
  - Gastrointestinal side effects<sup>3</sup>
  - Reduced bone mass<sup>4</sup>
  - Two-thirds stop GLP-1 therapy within 1 year<sup>5</sup>
  - Two-thirds of lost body weight returns within 1 year of stopping GLP-1 therapy<sup>5,6</sup>

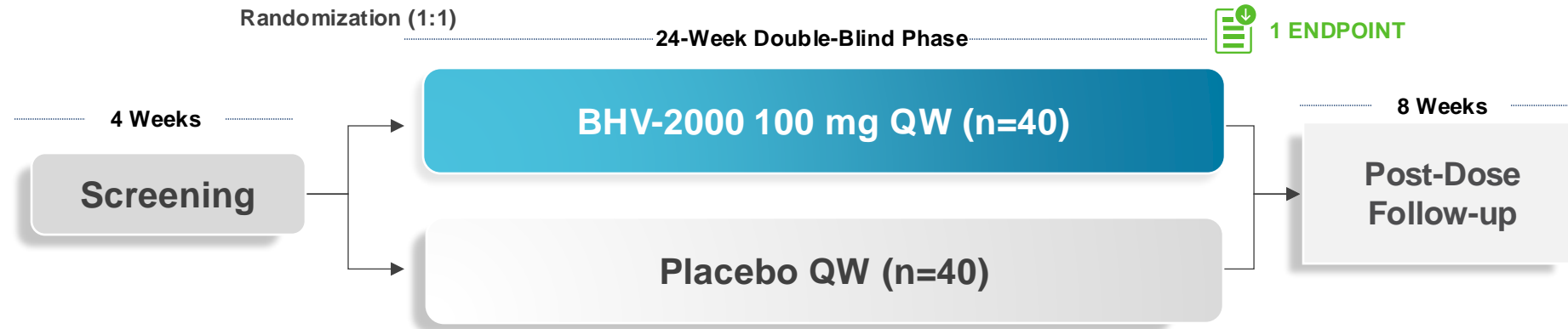
1 <https://www.worldobesity.org/resources/resource-library/world-obesity-atlas-2022>; Accessed 9-JAN-2025.  
2. Wilding JPH et al, *N Engl J Med*. 2021;384(11):989-1002. 3. Wilding, et. al., *Diabetes Obes Metab*. 2022; 24(8):1553-64. doi: 10.1111/dom.14725 4. Hansen MS, et al., *eClinicalMedicine*. 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.

## Complications of Obesity<sup>7</sup>





# Taldefgrobep Phase 2 Study in Obesity



<b>DESIGN</b>	Randomized, double-blind, placebo-controlled trial
<b>POPULATION</b>	Male and female adults living with overweight or obesity (BMI 27 - 40) without comorbid diabetes mellitus
<b>SAMPLE SIZE</b>	80 participants randomized 1:1 (Sex [M/F] and BMI [<35, ≥35-40])
<b>TREATMENT</b>	Taldefgrobep 100 mg SC QW via autoinjector vs. Placebo SC QW
<b>TREATMENT DURATION</b>	24-week treatment period, 8-week post-dose follow-up
<b>KEY ENDPOINTS</b>	Change in lean mass, fat mass, bone density, total body weight, and insulin sensitivity; PK/PD; safety/tolerability

**KEY  
POINT**

Phase 2 study planned to initiate in 1H 2025

# Company Capitalization Updates

## POTENTIAL ROYALTIES

Pfizer will make royalty payments in low- to mid-teens% in respect of annual US net sales of rimegepant and zavegepant >\$5.25B, subject to annual cap (\$400M/year)<sup>1</sup>

DAYS  
MATTER™

SHARES  
OUTSTANDING

CASH

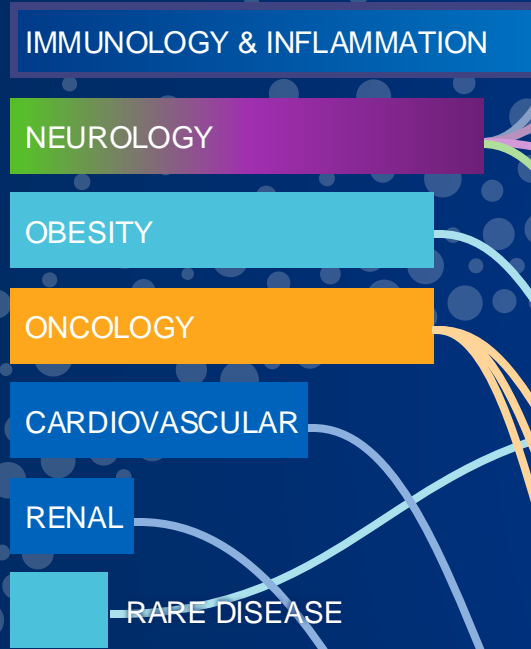
CGRP

101.1M<sup>2</sup>

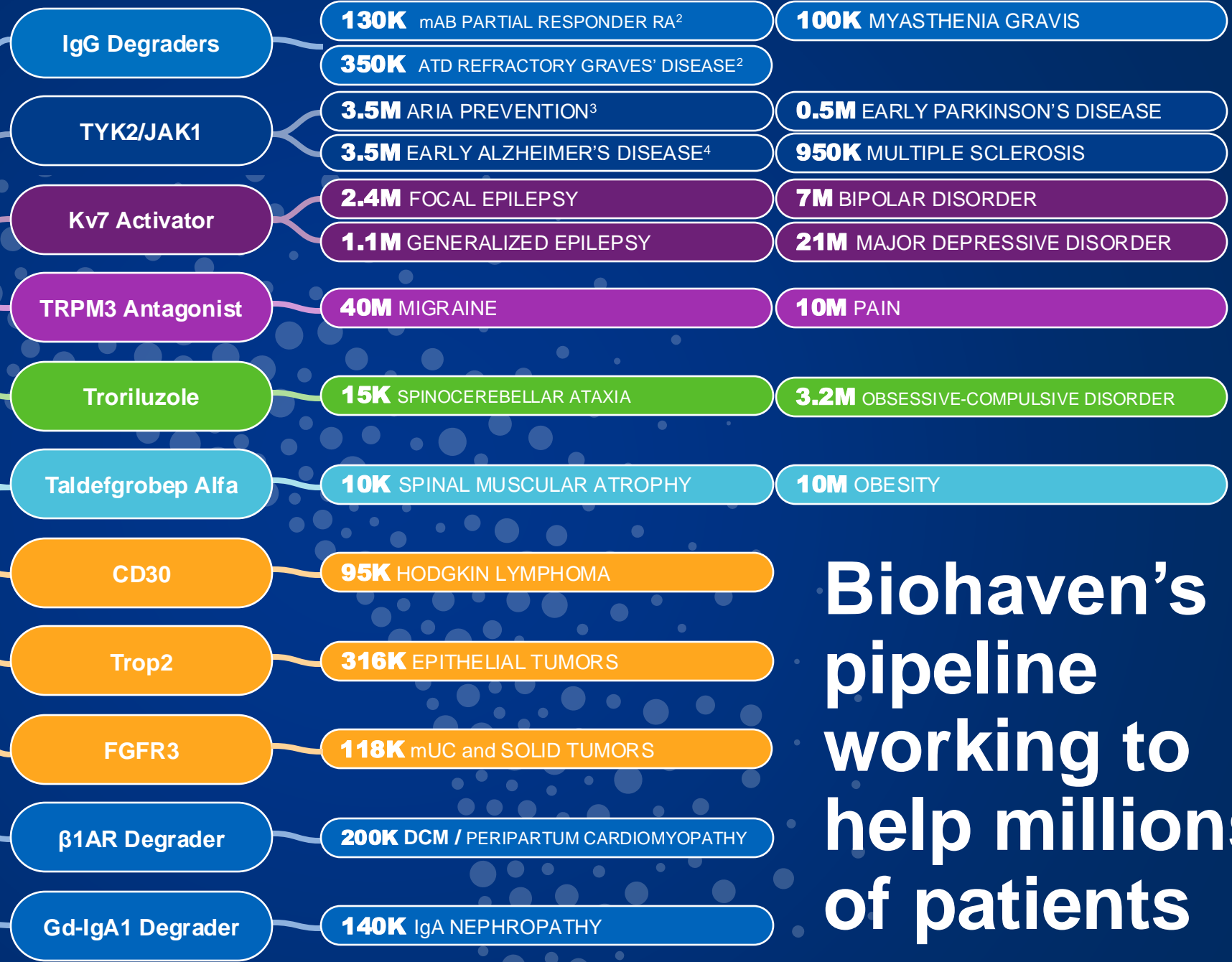
~\$642M<sup>3</sup>

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 November 8, 2024; excludes outstanding options. 3. As of October 2, 2024; includes proceeds raised from underwritten public offering

# Top Areas of Innovation



**biohaven**<sup>®</sup>



**Biohaven's pipeline working to help millions of patients**

1. Patient numbers are US from Biohaven market research; 2. represents ~12% and ~11% of Graves and RA populations respectively; 3. With amyloid therapy; 4. Disease modifying.

DAYS  
MATTER™

biohaven®