



Breaking Through Barriers in Neurology and Gene Therapy

Corporate Deck | January 2023

Forward-looking statements

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Voyager Therapeutics (Nasdaq: VYGR) Investment Thesis

Breaking Through Barriers in Neurology and Gene Therapy

CAPSID PLATFORM

The TRACER™ AAV Difference

- Potential to address gene therapy's narrow therapeutic window: superior BBB penetration at low doses
- Receptor identification supports potential for human translation
- Alliances with gene therapy leaders: Pfizer, Novartis

CNS PIPELINE

Capsids Plus Diverse Payloads

- Validated targets and biomarkers allow potential for rapid proof-of-biology
- Indications with high unmet need: ALS, Alzheimer's, Parkinson's, Friedreich's Ataxia
- Nominating lead candidates; IND filings expected 2024/2025

PARTNERSHIPS

Generating Non-Dilutive Revenue

- Capsid license structure exclusive to target, NOT capsid – enables multiple licenses
- Pfizer exercised option; Novartis decision expected Mar 2023
- Neurocrine: Collaboration on GBA1, FA, and five undisclosed targets
- Exploring additional partnerships

Breaking News: Voyager strategic collaboration with Neurocrine for GBA1+

VOYAGER RECEIVES:

Strengthens balance sheet

Up-front consideration of \$175M (\$136M cash, \$39M equity purchase at 50% premium)

Secures program funding

Program costs fully reimbursed*

Transformational downstream value

Up to \$4.2B in potential milestones (\$1.5B development, \$2.7B commercial) + **royalties [%]**

- GBA: U.S. low double-digit to twenty; ex-U.S. high single-digit to mid-teen
- Rare CNS targets: U.S. high single-digit to mid-teen; ex-U.S. mid single-digit to low double-digit

Option to elect 50/50 cost/profit sharing in U.S.
for GBA1 program following Phase 1



NEUROCRINE RECEIVES:

Worldwide rights to Voyager's GBA1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases* and three gene therapy programs directed to rare CNS targets, each enabled by Voyager's next-generation TRACER™ capsids, as well as a Board seat.

Collaboration has compelling strategic and financial rationale for Voyager

| | |
|---------------------------|---|
| Recognizes Pipeline Value | Underscores the value of a Voyager prioritized pipeline program; powerful combination of novel TRACER™ capsid and novel payload |
| Progress for Patients | Leverages Neurocrine's neuroscience R&D and commercial capabilities to improve the likelihood of reaching and helping patients |
| Partnership Momentum | Demonstrates Voyager's continuing success in capsid licensing, co-development collaborations and other partnerships |
| Attractive Financials | Provides transformational value; strengthens balance sheet capacity by providing near-term cash and maintains long-term value |
| Enables Voyager Growth | Provides resources to further advance Voyager's platform, prioritized pipeline programs, and additional cutting-edge research |

Collaboration demonstrates how Voyager is enabling neuro genetic medicine

NOVEL CAPSIDS

- IV-delivered
- 100-1000X CNS transduction
- Multiple CNS cell tropisms (neurons, glial cells); liver/DRG detargeting
- Capsid receptor identification

- ✓ Pfizer license option exercised
- ✓ Novartis license option election March 2023
- ✓ Neurocrine collaborating on multiple targets



DIVERSE PAYLOADS

- CNS diseases
- CNS targets
- Preclinical models
- Vectorizing payloads (siRNA, antibodies, gene replacement)

- ✓ Neurocrine collaborating on GBA1 and FA programs plus five discovery programs
- ✓ Voyager advancing wholly-owned programs in gene replacement, silencing, antibodies



ENABLING NEURO-GENETIC MEDICINE

OPTIMAL CAPSID



OPTIMAL PAYLOAD



PROGRAM VALUE CREATION

TRACER™ AAV Capsid Platform

The TRACER™ AAV Difference

Delivery will enable the future of neuro genetic medicine

DELIVERY currently limits **NEUROLOGY**

Difficult to deliver across blood-brain barrier (BBB)



DELIVERY currently limits **GENE THERAPY**

Narrow therapeutic windows with local or systemic delivery

NEURO DELIVERY of **GENETIC MEDICINES** **COMBINES THESE CHALLENGES**

IV dosing: Low BBB penetration. Weak CNS transduction.

Local dosing (IT, IP*): Steep gradients. Restricted penetration within brain.

VOYAGER IS ENABLING DELIVERY of **NEURO GENETIC MEDICINES**

I.V., CNS-tropic capsids →
high potency at low doses

Receptor ID → rational
design; may enable transport

Expertise vectorizing payloads
(antibodies, siRNA, etc.)

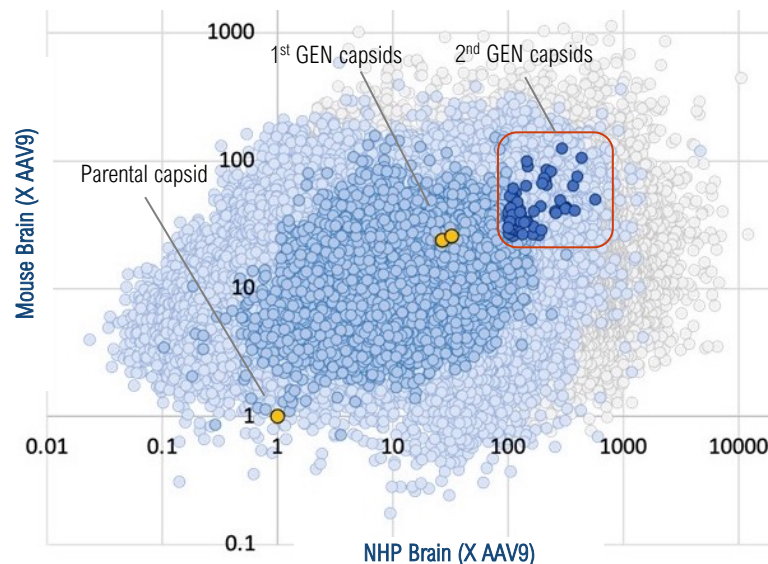
Neuropharmacology expertise
(diseases, targets, models)

Voyager's novel TRACER™-derived capsids power next-gen gene therapy

TRACER™ capsid discovery platform derived from evaluation of 200M+ variants of AAV5 and AAV9

- ✓ Superior BBB penetration across multiple species (mice and NHPs)*
- ✓ Enhanced neuronal and glial cell tropisms*
- ✓ Cross species transduction and receptor identification support human translation potential
- ✓ Broader therapeutic windows and de-targeting of undesired tissues (liver, DRG)*
- ✓ Selected by large pharma partners; enabling other external development opportunities
- ✓ TRACER™-derived capsids support internal pipeline programs

>100-fold improved CNS delivery across species



Data Presented at ASGCT 2022, Moyer et. al.

Voyager stands out in crowded CNS gene therapy space



Minimally invasive
I.V. Delivery



Receptor identification
enables rational design



Improved, broad
CNS transduction



Customizable cell tropisms
(neurons, glial cells) and
levels of liver de-targeting



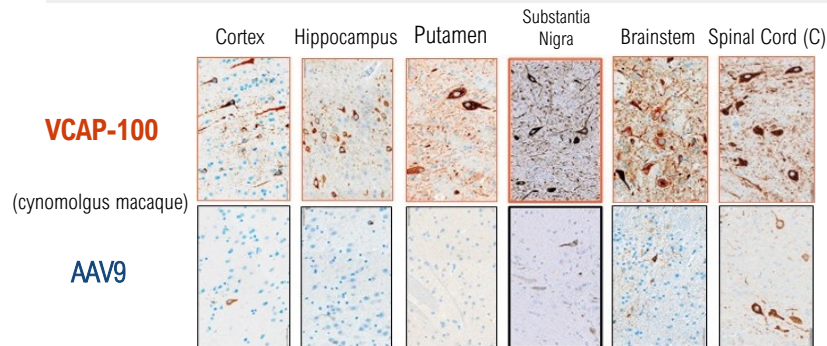
**Multi-Species
Validation**



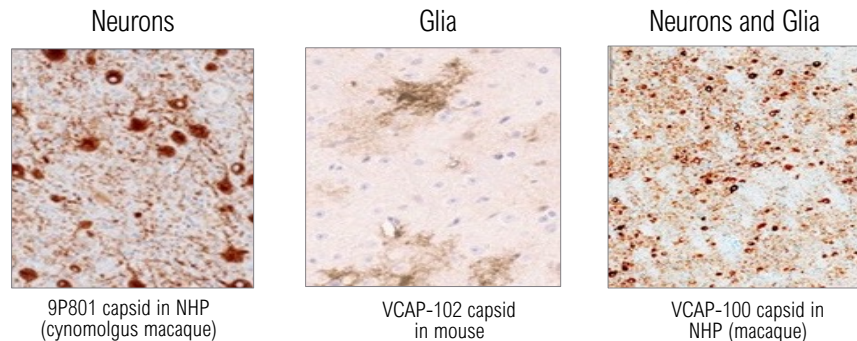
Fully integrated:
capsid engineering, NHP in vivo
validation, scalable production
(HEK, Sf9)

Novel IV delivered capsids with potential to transform CNS treatment

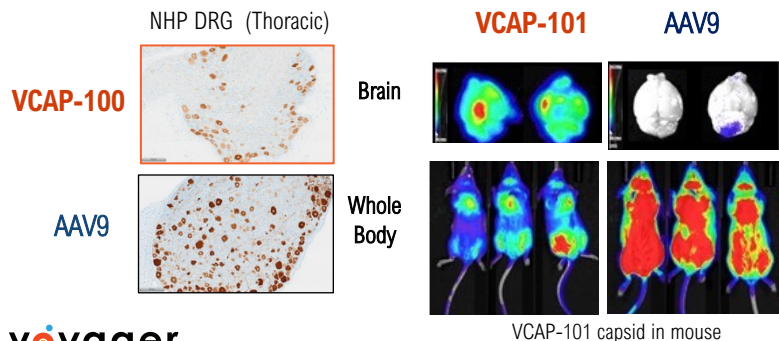
Widespread CNS distribution



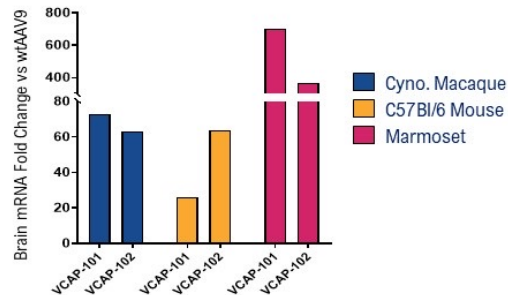
CNS neurons and glia transduced



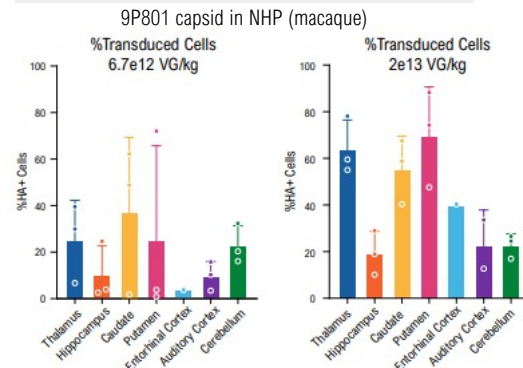
Detargeting of peripheral tissues



Cross species transduction



Transduction at low doses

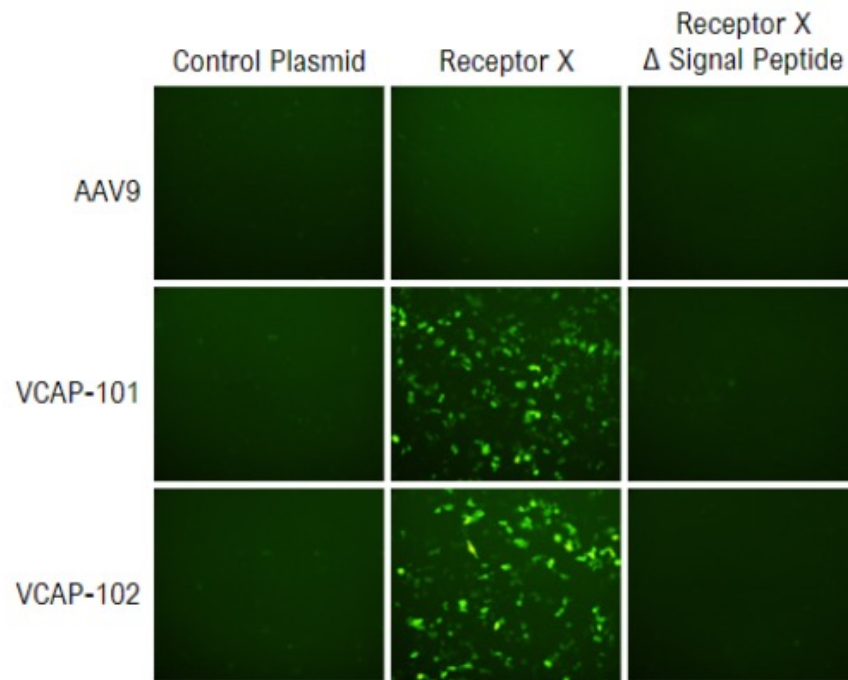


Receptor identified for TRACER™ capsid family

- ✓ Receptor identified for one of our most promising TRACER™ AAV capsids (ESGCT 2022)
- ✓ Expression confirmed in human endothelial cells and multiple CNS cell types

Characterizing the applicable receptor and confirming activity with the human orthologue increases probability that the related capsid will cross the BBB in humans

- Should facilitate rational design of AAV capsids for targeted IV delivery
- May enable IV delivery to CNS for diverse therapeutic modalities (preclinical experiments underway)



ESGCT 2022: Identification of a Cell Surface Receptor Utilized by an Engineered BBB-Penetrant Capsid Family with Enhanced Brain Tropism in Non-Human Primates and Mice

Transformative CNS Pipeline

Combining capsids with diverse payloads

CNS pipeline focuses on validated targets with high potential value

| Program (Mechanism) | Ownership | Early Research | Late Research | IND-Enabling |
|---|---|--------------------------|---------------|--------------|
| ALZHEIMER'S DISEASE Passive Tau Antibody | Wholly-Owned | | | |
| PARKINSON'S / OTHERS GBA1 Gene Therapy (Gene Replacement) | Neurocrine Collaboration (VYGR has 50% cost/profit split option)* | | | |
| ALS SOD1 Gene Therapy (Gene Silencing) | Wholly-Owned | | | |
| EARLY RESEARCH PROGRAMS Allele-specific mHtt + MSH3 gene silencing for HD; vHER2 antibody for brain mets | Wholly-Owned | | | |
| FRIEDREICH'S ATAXIA FXN Gene Therapy (Gene Replacement) | Neurocrine Collaboration (VYGR has 40% cost/profit split option)** | | | |
| UNDISCLOSED DISEASES / Five Gene Therapy Programs | | Neurocrine Collaboration | | |
| RARE NEUROLOGICAL DISEASE / Gene Therapy | | Pfizer License | | |
| CNS DISEASES / Three Gene Therapy Programs | | Novartis Option | | |

* After the Phase 1 readout, Voyager has the option to either: (1) co-develop and co-commercialize with Neurocrine Biosciences in the U.S. under a 50/50 cost- and profit-sharing arrangement, or (2) grant Neurocrine Biosciences full U.S. commercial rights in exchange for milestone payments and royalties based on U.S. sales.

**After the Phase 1 readout, Voyager has the option to either: (1) co-develop and co-commercialize with Neurocrine Biosciences in the U.S. under a 60/40 cost- and profit-sharing arrangement (Neurocrine/Voyager), or (2) grant Neurocrine Biosciences full U.S. commercial rights in exchange for milestone payments and royalties based on U.S. sales.

Anti-tau antibody offers a new twist on an Alzheimer's target

HIGH UNMET NEED + COMMERCIAL POTENTIAL

~6 million people in U.S.*

Multiple approaches

needed: Like oncology, combination treatment may improve outcomes (i.e. anti-amyloid + anti-tau)

VALIDATED TARGET

Tau

Pathology closely correlates with disease progression and cognitive decline

Targets C-terminal domain

Failed approaches targeted N-terminal; more consistent than mid-domain

EFFICIENT PATH TO PROOF-OF-BIOLOGY

New Tau PET tracers enable imaging for tau pathology and use as clinical trial biomarkers

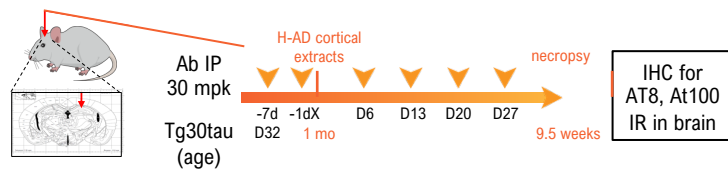
ROBUST PRECLINICAL PHARMACOLOGY

Voyager IV-delivered antibody inhibited spread of pathological tau by >70% in mouse seeding model (AAIC 2022)

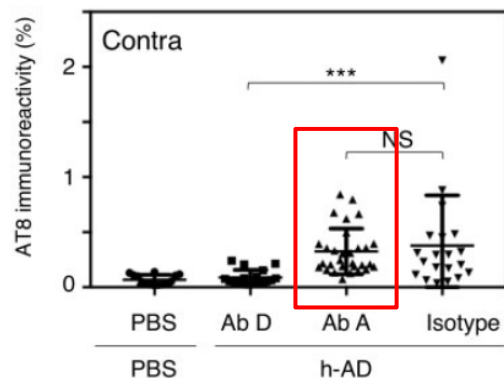
STATUS: Lead optimization underway

MILESTONE: ID lead candidate projected in H1 2023

Voyager's anti-tau antibody is differentiated from other anti-tau antibodies

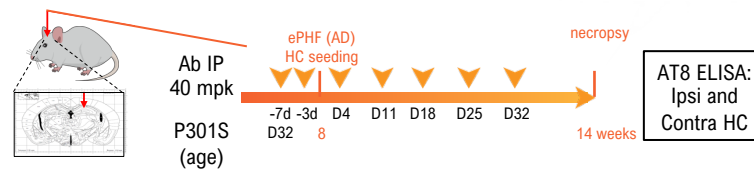


N-terminal Ab IPN002 is **not effective** in both mouse seeding model and clinic

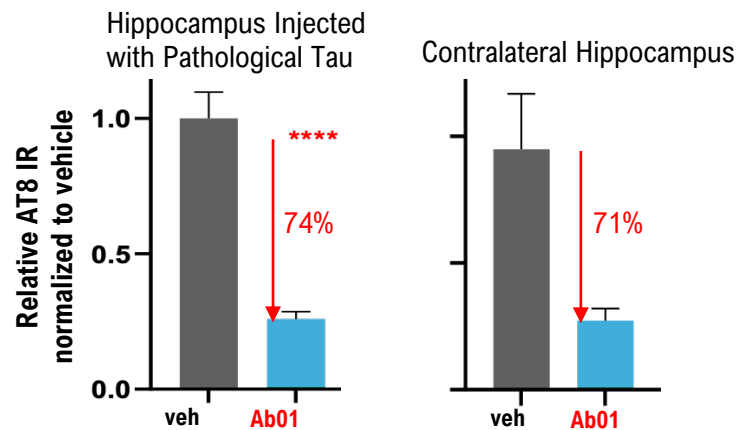


Note: Ab A targets N-terminus (aa15-24, IPN002)

Albert, *Brain*, 2019



Ab01 inhibits spread of pathological tau in mouse seeding model



Liu, *AAIC* 2022

GBA1 gene replacement; partnered with Neurocrine Jan 2023

HIGH UNMET NEED + COMMERCIAL POTENTIAL

~1 million people in U.S.*

>10% of PD patients
have a GBA1 mutation

Potential to treat
idiopathic PD

VALIDATED TARGET

GBA1

GBA1 mutations increase
the risk of PD ~20-fold*

STATUS:

Lead optimization
underway

EFFICIENT PATH TO PROOF-OF-BIOLOGY

GBA1 mutations
decrease expression
of GCase protein,
leading to substrate
elevation.

GCase and substrate
measurable in CSF

MILESTONE: ID lead candidate projected in H1 2023

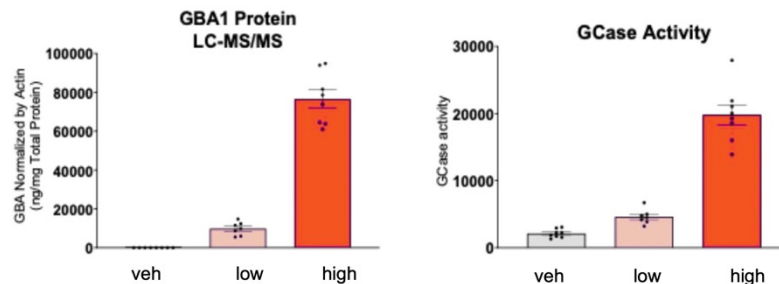
ROBUST PRECLINICAL PHARMACOLOGY

Preclinical data
demonstrate CNS target
engagement and delivery
of therapeutically relevant
levels of GCase in GBA
loss of function mouse
model.

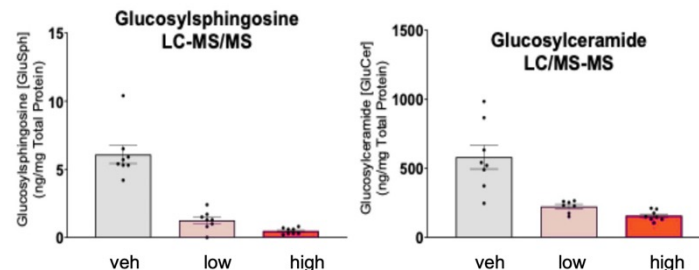
Restoration of GCase with IV-administered, BBB-penetrant AAV capsids

- BBB-penetrant AAV capsid provides *in vivo* proof-of-concept (POC) for increase in GCase with concomitant reduction of substrates
- Delivery of therapeutically relevant levels of GCase may disrupt disease process, and potentially slow neurodegeneration
- IV delivery with CNS-tropic capsid may enable widespread distribution to multiple affected brain regions and avoid need for more invasive approaches

GCase increased in GBA1 LOF mouse model



Substrate decreased in GBA1 LOF mouse model



Gene therapy approach to a validated target in ALS*

HIGH UNMET NEED + COMMERCIAL POTENTIAL

~20,000 people in U.S.**

~800 ALS patients have a SOD1 mutation

Incidence: 1 in 50,000**

Existing treatments are minimally effective; disease is typically fatal within 3 years of diagnosis

VALIDATED TARGET

SOD1

SOD1 mutations cause toxic gain of function in forms of familial ALS

Tofersen, under FDA review, is an ASO targeting SOD1 that has demonstrated clinical effect. Gene therapy approach could follow with more durable solution.

EFFICIENT PATH TO PROOF-OF-BIOLOGY

SOD1 measurable in CSF; plasma neurofilament light chain biomarkers measurable in plasma

ROBUST PRECLINICAL PHARMACOLOGY

Preclinical data showed robust SOD1 knockdown and significant improvements in motor performance and survival

STATUS: Lead optimization underway

MILESTONE: ID lead candidate projected in H1 2023

SOD1 knockdown approach shows preclinical survival benefit in mouse models

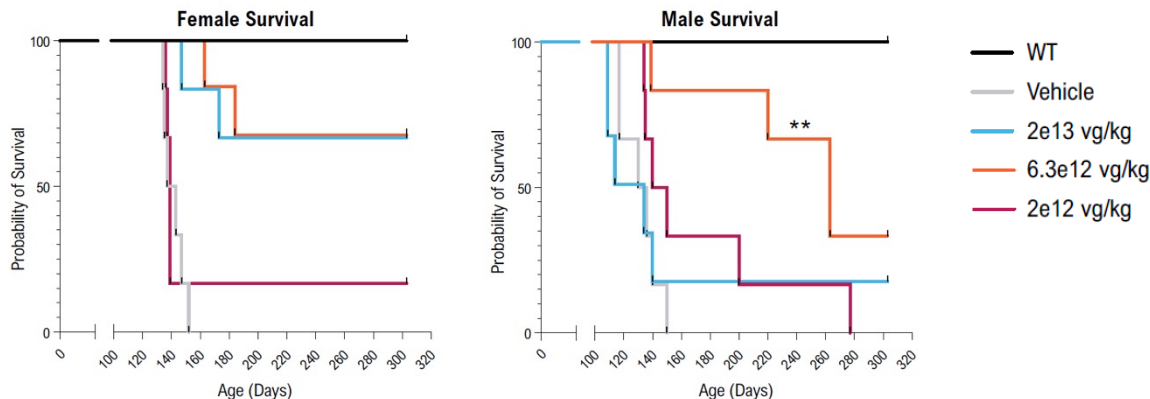
Strategy to combine highly potent siRNA construct with CNS-tropic, BBB penetrant TRACER capsid

- May enable broad CNS knockdown of SOD1, potentially addressing disease manifestations beyond the spinal cord

Promising preclinical results in mouse model

- Robust SOD1 knockdown in all levels of the spinal cord with IV dosing using a mouse BBB penetrant capsid
- Significant improvements in motor performance, body weight and survival

Increase in Survival



HD gene therapy initiative leverages latest in disease biology

HIGH UNMET NEED + COMMERCIAL POTENTIAL

~41,000 people in U.S.*

Incidence: 0.7 in
100,000**

Currently no cure or
treatment that can halt,
slow or reverse HD*

VALIDATED TARGETS

Allele-specific mHTT

Target the mutant protein
while preserving the
healthy version, which may
improve safety profile

MSH3

DNA repair enzyme
potentially involved in
harmful DNA expansions in
the HTT gene

EFFICIENT PATH TO PROOF-OF-BIOLOGY

Leveraging emerging
fluid-based
biomarkers and
imaging

ROBUST PRECLINICAL PHARMACOLOGY

First-in-class approach
based on evolving
research on the role of
somatic expansion in HD

STATUS:

Voyager is developing a vectorized
siRNA approach to silence HTT allele-
specifically and MSH3

MILESTONE:

Early research initiative to determine if
advancement warranted

Partnerships

Track Record of Non-Dilutive Revenue

Multiple partnership structures driving value



CAPSID LICENSES

PROVIDE:

- Validation (third-party buy-in, potential program use)
- Near-term funding (upfront and option exercise payments)
- Long-term funding (milestones and royalties)
- Multiple 'shots on goal' to prove human translation



PROGRAM PARTNERSHIPS

PROVIDE:

- Significant long-term potential value (profit share or milestones and royalties)
- Cost savings (program funding)
- Validation (investment into program)
- Near-term funding (upfront, early development milestone payments)







CREATIVE COLLABORATIVE STRUCTURES

PROVIDE:

- Opportunities to combine TRACER capsids and receptor technology with cutting-edge payloads and biologics, placing Voyager at forefront of neurogenetic medicine
- Various deal structures with range of participation being explored

DISCUSSIONS ONGOING

Existing partnership highlights

| | Disease/Target (Cells, Tissues, Transgenes) | Upfront Payment | Potential Option + Option Exercise Fees | Potential Development + Commercial Milestone Payments | Tiered Royalties |
|--|---|---|---|---|---|
|  NEUROCRINE BIOSCIENCES | GBA1 Program + 3 undisclosed targets (subject to HSR clearance) | \$175 million (\$136 million cash; \$39 million equity) | N/A | \$4.2 billion | GBA, U.S. low double-digit to twenty; ex-U.S. high single-digit to mid-teen. Rare CNS targets, U.S. high single-digit to mid-teen; ex-U.S. mid single-digit to low double-digit |
|  NOVARTIS | 3 undisclosed CNS targets (expandable to 2 additional rare CNS targets) | \$54 million | \$98.5 million | \$1.5 billion | Mid- to high-single-digit |
|  Pfizer | 1 undisclosed rare neurologic disease target | \$30 million | \$10 million – exercised | \$290 million | Mid- to high-single-digit |
|  NEUROCRINE BIOSCIENCES | Friedreich's Ataxia + 2 undisclosed targets | \$165 million (\$115 million cash; \$50 million equity) | N/A | \$1.3 billion | High-single-digit to high-teens in the U.S. and mid-single-digit to mid-teens ex-U.S. |

\$64 MILLION in 2022 payments extended cash runway into 2024;
Initial Novartis option exercise decision March 2023

Summary

Management team brings neurology and gene therapy expertise



Al Sandrock, M.D., Ph.D.
Chief Executive Officer



Robin Swartz
Chief Operating Officer



Todd Carter, Ph.D.
Chief Scientific Officer



Peter Pfreundschuh
Chief Financial Officer



Allen Nunnally
Chief Business Officer



Michelle Quinn Smith
Chief Human Resources Officer



Robert Hesslein
General Counsel



Trista Morrison
SVP Corporate Affairs



Recent Highlights and Upcoming Milestones

| | | |
|---------|-------------|---|
| H2 2022 | ✓ | Expanded team and BOD: CFO, CSO, SVP Corporate Affairs, BOD |
| H2 2022 | ✓ | Data at AAIC and ESGCT: novel tau-antibodies inhibit spread; low dose capsid/receptor data |
| Q3 2022 | ✓ | Pfizer option exercised on rare neurology target; \$10M payment |
| Q1 2023 | ✓ | Neurocrine strategic collaboration: potential \$4.4B for GBA1+ 3 discovery-stage programs |
| Q1 2023 | ○ | Novartis option exercise decision expected (potential for up to \$37.5M payment) |
| H1 2023 | ○ ○ ○ | Expect to ID lead candidates for all three priority pipeline programs: <ul style="list-style-type: none">• Tau antibody for Alzheimer's disease• GBA1 Parkinson's disease gene therapy• SOD1 ALS gene therapy |
| ONGOING | ○ | Potential for additional value-creating partnerships; discussions ongoing |



investors@vygr.com

voyagertherapeutics.com

