Forward-Looking Statements

This presentation contains forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward looking statements. For example, all statements we make regarding the length of our cash runway, the initiation, timing, progress and results of our preclinical studies and clinical trials and our research and development programs, our ability to advance our preclinical AAV-based gene therapies into, and successfully complete, clinical trials, our ability to continue to develop our product engine, and the timing or likelihood of regulatory filings and approvals, are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as updated by our future filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

This presentation also contains estimates and other statistical data made by independent parties and by the Company relating to market size and growth and other data about the Company's industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of the Company's future performance and the future performance of the markets in which the Company operates are necessarily subject to a high degree of uncertainty and risk.
Experienced Management Team
and founders that pioneered significant advances in AAV gene therapy and neuroscience, extensive CNS drug development expertise

Advancing Pipeline
Six programs targeting severe neurological diseases

Vector Engine
Robust product engine to engineer, optimize, manufacture and deliver AAV gene therapies

Scalable Manufacturing
Research grade and cGMP baculovirus/Sf9 production system

Strategic Collaboration
Sanofi Genzyme, ~$100 million upfront & up to $745 million in potential option and milestone payments
Voyager Product (Vector) Engine

Robust and Proprietary Capabilities Generating Novel Therapeutics

AAV Capsid Design & Selection
Tropism for relevant tissue and cell types

Vector Genome Design
Transgene selection for potent and selective pharmacology in target tissue

Manufacturing
Research and cGMP grade baculovirus / Sf9 production system

Delivery Optimization
Translatable dosing paradigm that provides target distribution in relevant tissues
Treating Severe Neurological Diseases with AAV Gene Therapy

Why CNS/Neurology?

• Significant unmet medical need
• Genetically-validated targets
• Targeted delivery to regions of the brain & broader delivery to the spinal cord is achievable
• Durable transgene expression as CNS cells are terminally differentiated
• Immune-privileged site

Why AAV?

• Ability to target a variety of tissue & cell types within the CNS
• >1,300 patients (>200 in CNS) treated, no AAV-related SAEs to date
• AAV does not readily integrate into the target cell genome, reducing potential for oncogenesis
• Ability to manufacture at commercial quality and scale
Commercial Scale AAV Manufacturing Capabilities

**Proprietary Process R&D**
- Process R&D center at Voyager’s headquarters
- Research grade baculovirus / Sf9 production system
- Up to 250L bioreactor capacity
- Proprietary reagents for new capsids and constructs

**Commercial Scale Capacity**
- Collaboration with MassBiologics and other CMO relationships
- cGMP baculovirus / Sf9 production system
- Up to 1,000L bioreactor capacity
- Voyager retains IP and key process know-how
Leverage different routes of administration and advances in dosing techniques to optimize delivery of our AAV vectorized antibodies.

<table>
<thead>
<tr>
<th>Routes of Administration</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Intraparenchymal Injection via Intra-operative MRI</td>
</tr>
<tr>
<td>2. Intravenous Injection with BBB penetrant AAV</td>
</tr>
<tr>
<td>3. Intrathecal Injection</td>
</tr>
<tr>
<td>4. Intramuscular Injection provides an mAb depot with single injection</td>
</tr>
</tbody>
</table>
Novel AAV Capsid Development Includes Potential for CNS Delivery with IV Injection

Greater GFP-IR in Mouse CNS after IV VOY-AAV101 vs AAV9

AAV9.GFP

VOY-AAV101

AAV9.GFP

VOY-AAV101

30-50X Higher Vg in Mouse Brain (Cortex, Brainstem, Striatum, Hippocampus, Cerebellum) and Spinal Cord after IV VOY-AAV101 vs AAV9
Experienced Leadership Team

Steve Paul, M.D. – President & CEO

Bernard Ravina, M.D.
Chief Medical Officer

Guangping Gao, Ph.D.
Leading AAV researcher, pioneered identification of new AAV serotypes

Krys Bankiewicz, M.D., Ph.D.
Translational neurosurgeon, AAV gene therapy pioneer

Dinah Sah, Ph.D.
Chief Scientific Officer

Mark Kay, M.D., Ph.D.
Leading researcher in AAV biology, capsid identification, RNAi

Kathleen Hayes
VP of Human Resources

Phil Zamore, Ph.D. – Leader & innovator in RNAi, including expressed RNAi

Jane Henderson
SVP, Chief Financial Officer

John Connelly
VP of Program & Alliance Management

Bob Pietrusko, PharmD
SVP, Regulatory Affairs & QA

Scientific Founders

Kathleen Hayes

Steve Paul, M.D. – President & CEO

Jane Henderson

John Connelly

Bob Pietrusko, PharmD

© Voyager Therapeutics
## 2017 Pipeline Programs

<table>
<thead>
<tr>
<th>Program</th>
<th>Preclinical</th>
<th>Lead Candidate Selection</th>
<th>Phase 1</th>
</tr>
</thead>
<tbody>
<tr>
<td>VY-AADC</td>
<td>Advanced Parkinson's Disease</td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-SOD101</td>
<td>Monogenic form of ALS</td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-HTT01</td>
<td>Huntington's Disease</td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-FXN01</td>
<td>Friedreich's Ataxia</td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-TAU01</td>
<td>FTD(3) / Alzheimer's Disease</td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-NAV01</td>
<td>Severe, Chronic Pain</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

(1) Sanofi Genzyme has ex-U.S. options, (2) Sanofi Genzyme has ex-U.S. options and option to co-promote in the U.S. (3) FTD = Frontotemporal Dementia
## 2019+ Pipeline Programs

<table>
<thead>
<tr>
<th>Program</th>
<th>Preclinical</th>
<th>Lead Candidate Selection</th>
<th>Phase 1</th>
<th>Phase 2-3</th>
</tr>
</thead>
<tbody>
<tr>
<td>VY-AADC</td>
<td>Advanced Parkinson's Disease</td>
<td></td>
<td></td>
<td>LONG-TERM PHASE 1B DATA</td>
</tr>
<tr>
<td>VY-SOD101</td>
<td>Monogenic form of ALS</td>
<td></td>
<td></td>
<td>PRELIMINARY BIOMARKER AND CLINICAL DATA</td>
</tr>
<tr>
<td>VY-HTT01</td>
<td>Huntington's Disease</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-FXN01</td>
<td>Friedreich's Ataxia</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>VY-TAU01</td>
<td>FTD(3) / Alzheimer's Disease</td>
<td></td>
<td></td>
<td>ESTABLISH PROOF OF CONCEPT</td>
</tr>
<tr>
<td>VY-NAV01</td>
<td>Severe, Chronic Pain</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

---

(1) Sanofi Genzyme has ex-U.S. options, (2) Sanofi Genzyme has ex-U.S. options and option to co-promote in the U.S. (3) FTD = Frontotemporal Dementia
Lead Program

VY-AADC for Advanced Parkinson’s Disease
One & Done Treatment for Advanced Parkinson’s Disease

100,000 - 150,000 Advanced Parkinson’s Disease (U.S.)

Oral levodopa
- Significant fluctuating on-off time
- Cognitive side effects

Continuous levodopa delivery: Duopa, ND0612
- Invasive, continual delivery required
- Infusion-site infections
- Non-compliance
- Short-term data (<12 weeks)

Deep Brain Stimulation
- In-dwelling hardware requiring revision/replacement/adjustments overtime
- Awake during surgery (drawback)
- Infections

VY-AADC:
ONE-TIME treatment, durable (>6 month)
 improvement in motor symptoms, function and quality of life measures, reductions in daily oral levodopa and associated side-effects, no in-dwelling hardware, no persistent injection-site reactions
Goal of VY-AADC Gene Therapy – Restore Function

Increase AADC levels in the putamen to produce more dopamine to “turn back the clock” for patients with advanced disease.

PD Patient’s Motor Fluctuations Over Time

Honeymoon Period

Early PD

Moderate PD

Advanced PD

Predictable

Unpredictable

Dyskinesia

On-Time

Off-Time

L-DOPA doses

© Voyager Therapeutics
In healthy individuals, presynaptic neurons that project into the putamen use the AADC enzyme to convert oral levodopa to dopamine.

AADC = aromatic L-amino acid decarboxylase
Targeting the Putamen with VY-AADC

Introduce VY-AADC into healthy postsynaptic neurons in the putamen to produce dopamine

VY-AADC = Voyager’s AAV gene therapy encoding the human AADC enzyme
Accurate delivery of gene for AADC enzyme (VY-AADC) to the putamen using real-time, intra-operative MRI during surgery to visualize delivery.
Patients representative of advanced stages of Parkinson’s disease

<table>
<thead>
<tr>
<th></th>
<th>Cohort 1</th>
<th>Cohort 2</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Age</strong></td>
<td>57.4 (7.2)</td>
<td>58.4 (8.6)</td>
</tr>
<tr>
<td><strong>Sex</strong></td>
<td>1 Female, 4 Male</td>
<td>5 male</td>
</tr>
<tr>
<td><strong>PD Duration (years)</strong></td>
<td>9.9 (4.6)</td>
<td>10.1 (1.6)</td>
</tr>
<tr>
<td><strong>UPDRS II off</strong></td>
<td>13.6 (2.1)</td>
<td>16.0 (1.7)</td>
</tr>
<tr>
<td><strong>UPDRS II on</strong></td>
<td>3.0 (2.9)</td>
<td>3.6 (1.7)</td>
</tr>
<tr>
<td><strong>UPDRS III off</strong></td>
<td>37.2 (5.9)</td>
<td>35.8 (7.6)</td>
</tr>
<tr>
<td><strong>UPDRS III on</strong></td>
<td>7.6 (5.1)</td>
<td>17.0 (3.8)</td>
</tr>
<tr>
<td><strong>Avg. Diary off-time (hrs)</strong></td>
<td>4.9 (1.7)</td>
<td>4.2 (1.4)</td>
</tr>
<tr>
<td><strong>Avg. Diary on-time (hrs)</strong></td>
<td>10.5 (2.1)</td>
<td>10.7 (1.8)</td>
</tr>
<tr>
<td><strong>PDQ-39</strong></td>
<td>18.2 (10.2)</td>
<td>16.6 (12.7)</td>
</tr>
<tr>
<td><strong>Hoehn and Yahr Stage</strong></td>
<td>3.0</td>
<td>3.0</td>
</tr>
<tr>
<td><strong>LED(1) mg</strong></td>
<td>1467.5 (615.0)</td>
<td>1635.5 (687.3)</td>
</tr>
</tbody>
</table>

Mean (standard deviation)

(1) Levodopa equivalent dose
Interim Phase 1b Results Strengthens Clinical Hypothesis

Dose-Dependent and Time-Dependent Improvements

**Increased**
Putamen Coverage
C1 (21%)
C2 (34%)
C3 (42%)\(^1\)

**Increased**
AADC Expression
C1 (13%)
C2 (56%)\(^1\)

**Reduced**
L-DOPA Doses
C1 (10%)
C2 (35%)\(^2\)

**Improved**
Motor Signs-UPDRS-III On Medication
C1 (1.6-pt, 21% worsening)
C2 (9.6-pt 56% improvement)\(^1,3\)

**Improved**
Motor Signs- Diary
On-time increase
C1 (1.6 hrs, 16%)
C2 (4.1 hrs, 43%)\(^2\)

Off-time decrease
C1 (1.4 hrs, 27%)
C2 (2.4 hrs, 48%)\(^2\)

**Improved**
QOL (PDQ-39)
C1 (1.9-point improvement)
C2 (9.2-point improvement)\(^2\)

Note: “C1, C2” = Cohort 1, Cohort 2

1) Data at 6-months
2) Cohort 1 12-month data from 5/5 patients and Cohort 2 12-month data from 3/5 patients. 2/5 patients in Cohort 2 have not reached 12-month follow-up
3) Actual results reported at six months and were maintained at 12 months
Increased Coverage of the Putamen with VY-AADC

Coverage in Cohort 1 (21%), Cohort 2 (34%) and Cohort 3 (42%)

Note: % coverage of the putamen represents average coverage of left and right putamen by volume. Error bars are standard deviations. Source: Voyager Therapeutics, Inc. press release January 20, 2017
Dose-Related Increase in AADC Enzyme Activity

Cohort 1 (13%) and Cohort 2 (56%)

% Increase from baseline to six months in $^{18}$F-Dopa PET Signal with the putamen

Cohort 1 missing one scan at baseline
Meaningful Reduction in Dopaminergic Medication

35% reduction in daily oral dose of levodopa and related medications in Cohort 2 at 6 months

Mean +/- SE.  6-month data from 5/5 patients in each of Cohorts 1 & 2.  12-month data from 5/5 patients (Cohort 1) and 3/5 patients with 12 months of follow-up.  2/5 patients in Cohort 2 have not reached 12-month follow-up
4.1 hour (43%) increase in diary on-time at 12 months in Cohort 2

Diary times normalized to 16-hour waking day
Mean +/- SE. 6-month data from 5/5 patients in each of Cohorts 1 & 2. 12-month data from 5/5 patients (Cohort 1) and 3/5 patients with 12 months of follow-up. 2/5 patients in Cohort 2 have not reached 12-month follow-up.
Summary Patient Diary (12-months) – Better On-time

4.1 hour (43%) increase in on-time with concurrent 2.2 hour (48%) decrease in off-time in Cohort 2

Diary times normalized to 16-hour waking day
Mean +/- SE. 6-month data from 5/5 patients in each of Cohorts 1 & 2. 12-month data from 5/5 patients (Cohort 1) and 3/5 patients with 12 months of follow-up. 2/5 patients in Cohort 2 have not reached 12-month follow-up.
Improvements in Function and Quality of Life

*Cohort-Dependent Improvements at 12 months in Function, Activities of Daily Living, and Quality of Life*

<table>
<thead>
<tr>
<th>Cohort</th>
<th>UPDRS-II Off Medication</th>
<th>UPDRS-IV Total Score</th>
<th>PDQ-39 Total Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cohort 1</td>
<td>0.2 point worsening (SD 3.7)</td>
<td>1.2 point improvement (SD 1.9)</td>
<td>1.9 point improvement (SD 5.6)</td>
</tr>
<tr>
<td>Cohort 2</td>
<td>4.0 point improvement (SD 1.0)</td>
<td>2.7 point improvement (SD 3.1)</td>
<td>9.2 point improvement (SD 5.5)</td>
</tr>
</tbody>
</table>

Baseline Cohort 1 and Cohort 2 (from 3/5 patients) for UPDRS-II Off medication (13.6, 16.7, respectively), UPDRS-IV Total Score (7.8, 8.7, respectively) and PDQ-39 Total Score (18.2, 12.3, respectively)

12-month data from 5/5 patients (Cohort 1) and 3/5 patients with 12 months of follow-up. 2/5 patients in Cohort 2 have not reached 12-month follow-up.
VY-AADC Phase 1b Results: Safety

- Surgical procedure successfully completed in all 15 patients to date
- Infusions of VY-AADC01 have been well-tolerated with no vector-related serious adverse events (SAEs).
- 14 of the 15 patients were discharged from the hospital within two days following surgery.
- As previously reported, one patient experienced two SAEs - a pulmonary embolism or blood clot in the lung, and related heart arrhythmia or irregular heartbeat.
  - Patient treated with an anti-coagulant and symptoms associated with the SAEs have completely resolved.
  - Investigators determined that this was most likely related to immobility during the surgical procedure and subsequent formation of a blood clot, or deep vein thrombosis (DVT), in the lower extremity. Consequently, DVT prophylaxis was added to the surgical protocol and no subsequent events have been observed following implementation of these measures.
VY-AADC – Initiate Pivotal Phase 2-3 Program in Late 2017

Phase 2
- Double-blind, placebo-controlled (30-42 patients)
- Primary clinical endpoint: motor symptom improvement (Diary on- or off-time) at 12 months
- Putaminal coverage and PET imaging data

Phase 3
- Begins in staggered parallel, or prior to completion of Phase 2
- Global, double-blind, placebo-controlled (100-120 patients)
- Primary endpoint: motor symptom improvement (Diary on- or off-time) at 12 months
VY-AADC Near-Term Milestones

- Initiate pivotal, placebo-controlled program in advanced Parkinson’s disease (Q4:17)
- Sanofi-Genzyme opt-in decision (Q3:17)
- Provide Cohorts 1-3 Phase 1b results (Q3:17)
- Initiate posterior trajectory trial (Q2:17)
- Presented interim Phase 1b results at AAN/AANS
- Completed Cohort 3 enrollment

© Voyager Therapeutics
Additional Pipeline Programs
Devastating neurodegenerative disease: rapidly progressive, adult-onset, fatal, affecting ~20,000 patients in the U.S.

- Degeneration of nerve cells in spinal cord and brain results in severe muscle atrophy with loss of ability to walk and speak, usually fatal within 2-4 years of diagnosis
- 90% of cases are sporadic, 10% of cases due to genetic (familial) causes
- Mutation in superoxide dismutase 1 (SOD1) gene causes ~20% of familial cases and 1-2% of sporadic cases (400-800 patients)
  - Toxic gain-of-function mutation results in severe motor neuron pathology

Goal with VY-SOD101:

- Composed of AAV capsid and transgene that harnesses the RNAi pathway to selectively silence, or knock-down, the levels of SOD1 messenger RNA.
- With a single intrathecal (IT) injection, potential to durably reduce toxic mutant SOD1 protein in CNS to slow the progression of disease
- Potential to leverage for other monogenic forms (e.g., C9orf72, TDP-43/FUS) for an additional ~2,000 – 4,000 patients
ALS: Proof-of-Concept for AAV Gene Therapy

50-90% SOD1 Knockdown in NHP lumbar spinal cord after IT dosing of AAV9-shSOD1

Survival extension in mouse models of ALS after IV dosing of AAV9-shSOD1

Foust et al., 2013

Delivery to Dog Motor Neurons

Source: Voyager Therapeutics
VY-SOD101 Achieves Significant Knockdown of SOD1 in Preclinical Models

VY-SOD101 Knockdown in Non-Human Primate Motor Neurons

SOD1 mRNA Expression (Relative to Vehicle Average)

~75% knockdown achieved with VY-SOD101

P < 0.0001

IND-enabling studies underway. File IND: 4Q17/1Q18

Source: Voyager Therapeutics
Huntington’s Disease Program VY-HTT01: Overview and Goal

Fatal, inherited, neurodegenerative disease affecting ~70,000 patients in the U.S./EU

- Progressive decline of motor and cognitive functions and a range of behavioral and psychiatric disturbances.
  - Symptoms usually appear between ages of 30 to 50 and worsen over a 10 to 25-year period
- Caused by toxic gain-of-function mutation in the *huntingtin*, or HTT, gene, an autosomal dominant triplet repeat expansion (CAG) encoding poly-glutamine in N-terminus of HTT protein in all patients
- Mutations in HTT gene lead to abnormal intracellular huntingtin protein aggregates causing neuronal cell death

Goal with VY-HTT01:

- Composed of AAV capsid and transgene that harnesses the RNAi pathway to knockdown levels of mutant HTT messenger RNA
- One-time parenchymal delivery to brain regions relevant to Huntington’s disease - striatum and cortex
- Slow disease progression as measured by functional rating scales and supported by cognitive, behavioral, and motor measures

Professor Nancy Wexler and a boy with Huntington’s Disease, Venezuela, 1990s
Preclinical Data Supports Rationale for Targeting HTT

>50% knockdown of HTT results in significant functional benefit

55% Knockdown of HTT with AAV Ameliorates Rotarod Deficits in Mouse Model (YAC128)

55% Knockdown of HTT with AAV Normalizes Depressive Behavior in Mouse Model (YAC128)

Source: Stanek et al., 2014
VY-HTT01 Achieves Significant Knockdown of HTT in Preclinical Models

>50% knockdown of HTT Observed with VY-HTT01 Lead Clinical Candidate in Non-Human Primate

Group 1=VY-HTT01, Groups 1-4=different candidate pri-miRNA targeting HTT, Group 5=vehicle

IND-enabling studies underway. File IND: 2018

Source: Voyager Therapeutics
Fatal, debilitating neurodegenerative and cardiac disease affecting ~6,400 patients in the US:

- Progressive ataxia to wheelchair dependence, loss of sensation, cardiomyopathy, scoliosis and diabetes as well as impaired vision, hearing and speech.
- Typical age of onset is 10 to 12 years and life expectancy is severely reduced with patients generally dying of neurological and cardiac complications between 35 to 45 years of age.
- Mutations of FXN gene reduce production of frataxin protein resulting in degeneration of sensory pathways and debilitating symptoms.
- Autosomal recessive disorder - ~50% of normal frataxin protein levels may be sufficient to treat disease.

Goal with VY-FXN01:

Composed of AAV capsid and transgene encoding human FXN, with a single intrathecal or intravenous injection, to restore FXN protein levels to at least 50% of normal in relevant neurons and cardiac myocytes to slow the progression of disease.
Friedreich’s Ataxia Lead Candidate Optimization

Engineered promoters provide wide range of transgene expression in vitro

86-fold range of FXN expression in CNS

Lead candidate selection underway to optimize capsid, promoter, and FXN transgene

Source: Voyager Therapeutics
## Upcoming Milestones

<table>
<thead>
<tr>
<th>Milestone</th>
<th>Timing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Selected lead candidate VY-SOD101 for ALS and VY-HTT01 for Huntington’s disease</td>
<td>✓</td>
</tr>
<tr>
<td>Provide Cohort 1-3 and preliminary posterior trajectory study data for VY-AADC for advanced Parkinson’s disease</td>
<td>Q3:17</td>
</tr>
<tr>
<td>Select lead candidate for Friedreich’s ataxia program</td>
<td>2017</td>
</tr>
<tr>
<td>Sanofi-Genzyme opt-in decision for VY-AADC for advanced Parkinson’s disease</td>
<td>Q3:17</td>
</tr>
<tr>
<td>Initiate pivotal, placebo-controlled trial for VY-AADC in advanced Parkinson’s disease</td>
<td>Q4:17</td>
</tr>
<tr>
<td>File IND for VY-SOD101 for monogenic form of ALS</td>
<td>Late’17/Early’18</td>
</tr>
<tr>
<td>Provide longer-term Cohort 1-3 and preliminary posterior trajectory study data for VY-AADC for advanced PD</td>
<td>1H:18</td>
</tr>
<tr>
<td>Provide biomarker data for VY-SOD101 for monogenic form of ALS</td>
<td>2018</td>
</tr>
<tr>
<td>File IND for VY-HTT01 for Huntington’s disease</td>
<td>2018</td>
</tr>
</tbody>
</table>
Voyager Investment Highlights

Why Voyager?

- **Focused leader** in AAV gene therapy for severe neurological diseases

- **Proof-of-concept** established for VY-AADC01 for Parkinson’s disease: Pivotal program to begin **4Q:17**

- **Advancing pipeline**: 3 INDs planned <24 months
  - ALS/Huntington’s disease/Friedreich’s ataxia

- **Robust vector product** engine to regenerate pipeline
  - Anti-tau and Severe, Chronic Pain