Odylia THERAPEUTICS

Bringing life changing treatments to people with genetic diseases regardless of prevalence or commercial interest

re*Imagining* drug development for rare disease

A World re***Imagined***

Almost 7,000 rare diseases affect 400 million people worldwide - more than the entire United States population

Human costs are colossal

- Lost opportunities, wages, independence, experiences
- High societal costs
- Skyrocketing medical expenses
- Significant loss of life

Obstacles to finding a treatment can be significant

- Expensive research and development
- Slow progress
- Low financial return on investment

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Introduction to Odylia

About Odylia

Independent nonprofit organization founded in 2017

Mission

Utilize a unique, nonprofit business model to accelerate the development of gene therapies for people with rare disease, changing the way treatments are brought from the lab to the clinic

Vision

Bring life changing treatments to people with genetic disease regardless of prevalence or commercial interest

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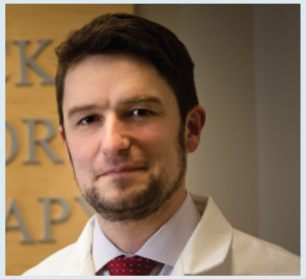
About Odylia Therapeutics, Inc.

Odylia Therapeutics

- Nonprofit biotech
- Founded through a collaboration between Mass Eye & Ear and Usher 2020 Foundation
- Funding from biopharma partnerships, philanthropy, and services
- Headquarters in Atlanta, Georgia

Odylia's Leadership Team



Luk Vandenberghe, PhD, Co-Founder

Assistant Professor, Harvard Medical School and Director, Grousbeck Gene Therapy Center, Massachusetts Eye and Ear. He has discovered and developed new technologies in the gene therapy field and started numerous companies to bring treatments to patients.

Scott Dorfman, Co-Founder & Chief Executive Officer

Prior to Odylia, Mr. Dorfman founded and was Chairman, & CEO of Innotrac Corporation (NASDAQ: INOC), which was a full-service provider of eCommerce technology, fulfillment, and call center services until combining with eBay Enterprises to form Radial Corporation. Scott currently serves as an Operations Partner for Fulcrum Equity Partners and sits on the Board of Dropoff Inc., Complemar Corporation, Fulcrum Equity, Odylia Therapeutics and Usher 2020 Foundation.

Ashley Winslow, PhD, Chief Scientific Officer

Ashley received her PhD in Medical Genetics from the University of Cambridge and completed her postdoctoral work at Massachusetts General Hospital and Harvard Medical School. Before joining Odylia, Ashley worked in the Precision Medicine and Human Genetics and Computational Biomedicine group at Pfizer and the Orphan Disease Center at the University of Pennsylvania.

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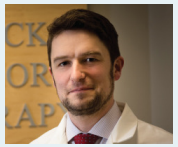
Board Members & Advisors

Board of Directors



Luk Vandenberghe, Ph.D.
Assistant Professor, Harvard Medical School
Director, Grousbeck Gene Therapy Center,
Massachusetts Eye and Ear

Scott Dorfman, B.B.A.
CEO, Odylia Therapeutics

Emil Kakkis, M.D., Ph.D.
President, CEO, and Founder
Ultragenyx Pharmaceutical Inc

Mat Pletcher, PhD
Senior Vice President, Head of Research
Audentes Therapeutics;
Board Member, The RDH12 Fund for Sight

Advisory Board

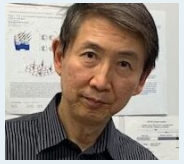
Eric Pierce, M.D., Ph.D.
Director, Ocular Genomics Institute
Harvard Medical School
Director, Inherited Retinal Disorders Services
Massachusetts Eye and Ear

Joy Cavagnaro, Ph.D.
President, Access Bio

Jean Bennett, M.D., Ph.D.
Professor, Perelman School of Medicine
University of Pennsylvania

Gregory Robinson, Ph.D.
Chief Scientific Officer

Alan Spiro, B.A., Ph.D., J.D.
Partner, ExSight Ventures



Tiansen Li, Ph.D.
National Eye Institute, NIH

Alberto Auricchio, Ph.D.
Associate Professor of Medical Genetics
“Federico II” University Napoli
Principal Investigator, TIGEM

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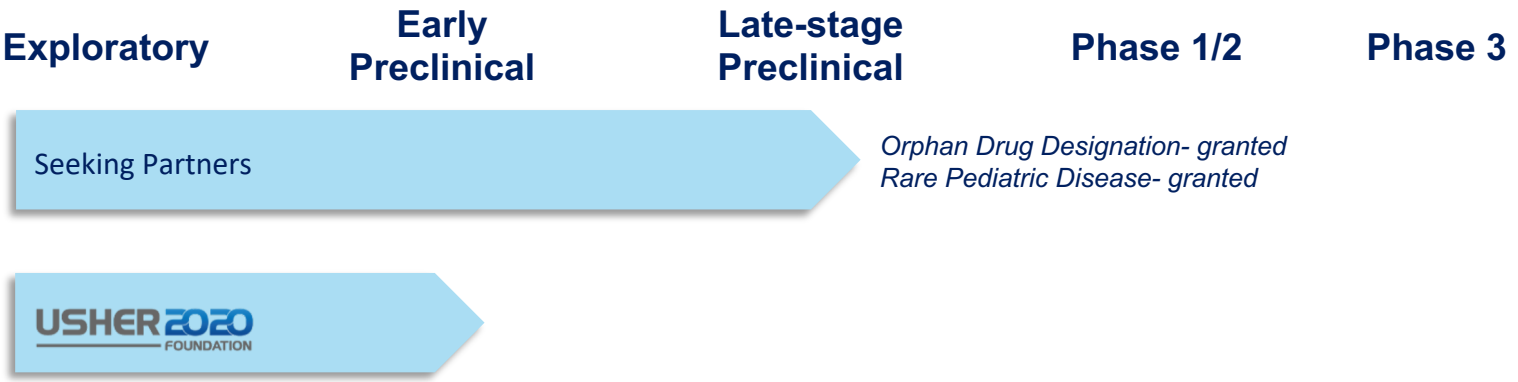
Rare Disease Portfolio

Gene Therapy Pipeline (disclosed)



Brydge Solutions- 2021 Patient Group Partnerships

SATB2 Gene Foundation	RDH12 Fund for Sight	Usher 2020 Foundation	CLOVES Foundation	Open Treatments
sat b2 gene foundation	THE RDH12 FUND FOR SIGHT	USHER 2020 FOUNDATION	CLOVES SYNDROME COMMUNITY	Open Treatments

Odylia's Origin Story

2011 — **Children Diagnosed with Rare Disease**
A family learned their children have a rare disease, Usher Syndrome (~350 people with disease in US) and they created a Patient Advocacy Group to fund study of the cause and possible treatments

2013 — **Research Funded**
Invested $5 million in various scientific research programs

2015 — **Major Obstacles Prevent Treatments from Moving to Clinical Trials**
A promising treatment was identified but there was no clear path or commercial partner to take it forward to clinical trials

2016 — **Work Toward Treatment Stalled**
After investing in three more therapeutic approaches and working with multiple researchers to move a treatment for their children forward, **work completely stalled.** There was no apparent way forward

2017 — **Lost Time and Money Inspires a New Paradigm and Odylia Therapeutics Founded**
Realized if they had a **strategic research plan** and know-how, they could have saved at least $3 million on science that was never going to lead to a treatment. A different model is needed. Odylia Therapeutics was founded as a nonprofit biotech to accelerate and streamline the development of treatments

2020 — **Odylia Enables Rapid Advancement Toward Treatment**
Odylia Therapeutics began work on the Usher Syndrome Program - created a strategic research plan and brought together key resources to advance program toward clinical trial. The program progressed **more in one year** than in the last five years without Odylia

2021 - 2022 — **Clinical Trials**
Odylia Therapeutics will complete work needed for regulatory submission of the USH1C Gene Therapy Program

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AAV Delivery: Anc80, a Novel Tool for Subretinal Delivery

Anc80 Tropism: retina, CNS, liver, muscle, cochlea

Anc80 capsid:

- Anc80 is an ancestral AAV serotype developed by Luk Vandenberghe's lab using ancestral sequence reconstruction; the predicted ancestor of AAV serotypes 1, 2, 8, and 9 and shows broad tissue tropism
- Superior expression & kinetics (NHP, pig, murine testing)
- Improved tropism for diverse cell types compared to other capsids
- 14-year patent life remaining (WO15054653)

Tropism- multiple tissues:

- Central Nervous system tropism in mouse: Hudry et al., 2018
- Retina: Zinn, 2015; Carvalho, 2017; Carvalho, 2018
- Liver: Zinn, 2015
- Muscle: Zinn, 2015
- Kidney: Ikeda, 2018
- Inner Ear: Landegger, 2015; Pan 2017; Suzuki, 2017; Tao, 2018

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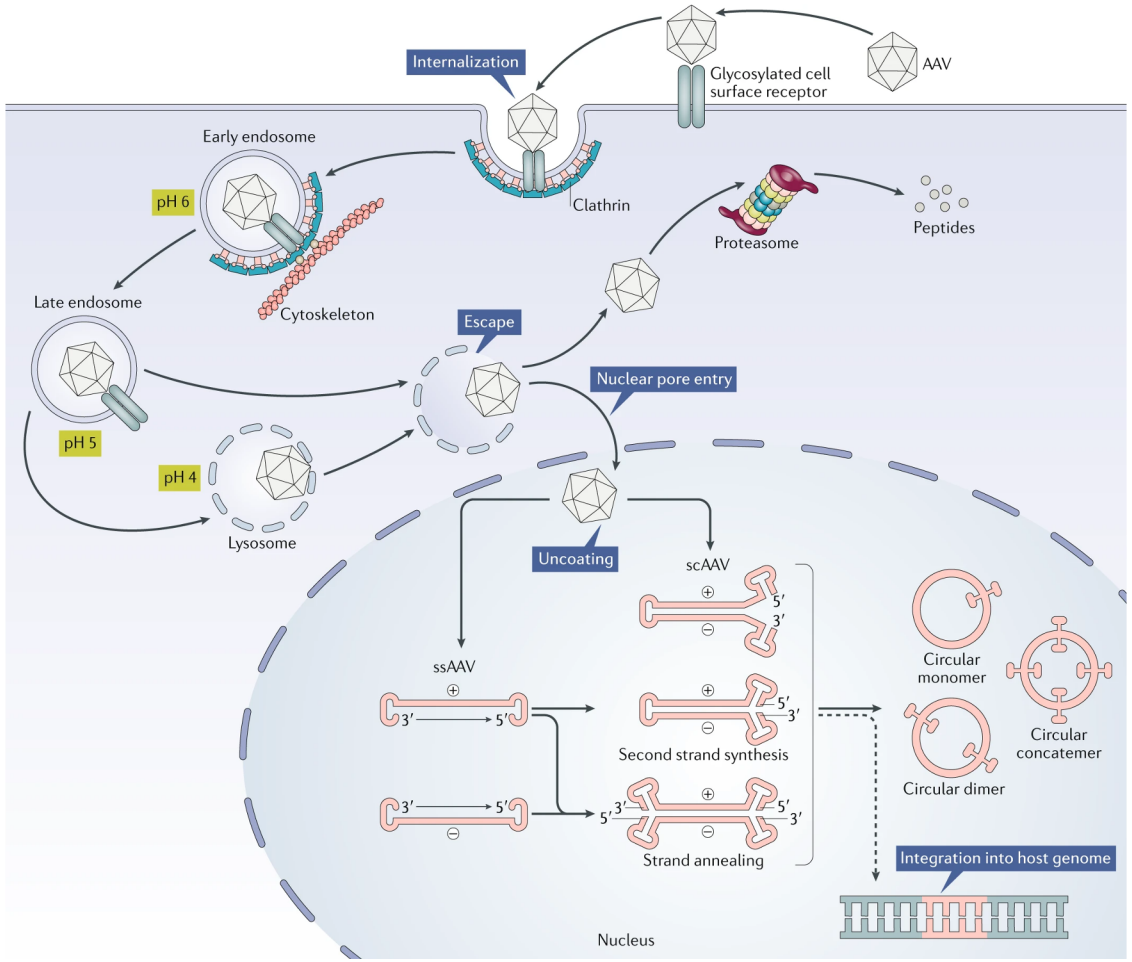
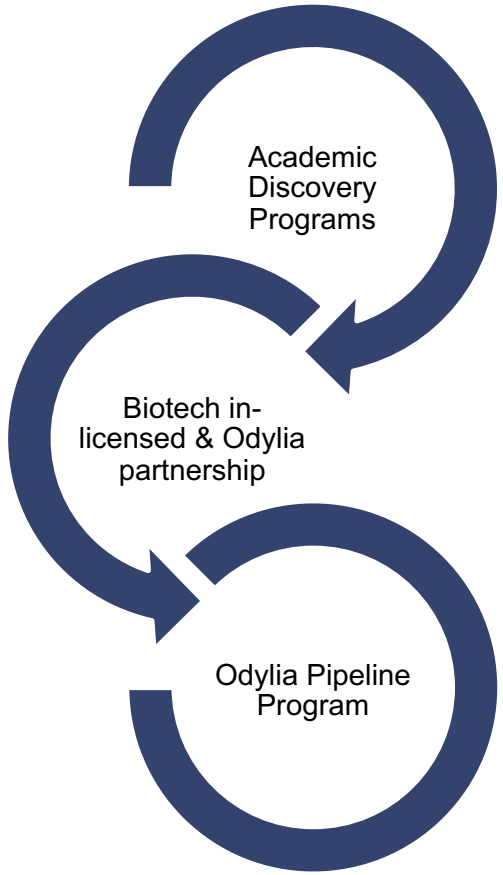
Anc80 AAV Vector

- Mass Eye & Ear developed Anc80 AAV technology
- Odylia licensed worldwide exclusively for rare retinal diseases
- Option to expand into additional genetic diseases and organ systems
- Sensorimotor (hearing) exclusively licensed to Akous
- PCT (WO/2015/054653) United States
- A highly favorable license. Sub-licensor to Odylia pays no license fee or royalties to ME&E



Bottlenecks in Drug development for Rare Disease:
RPGRIP1 case study

OT-004: RPGRIP1 Gene Therapy



2005- 2016

- Initial studies show early proof-of-concept, but lack of funding and resource focus to move the program forward
- Program moves to Odylia

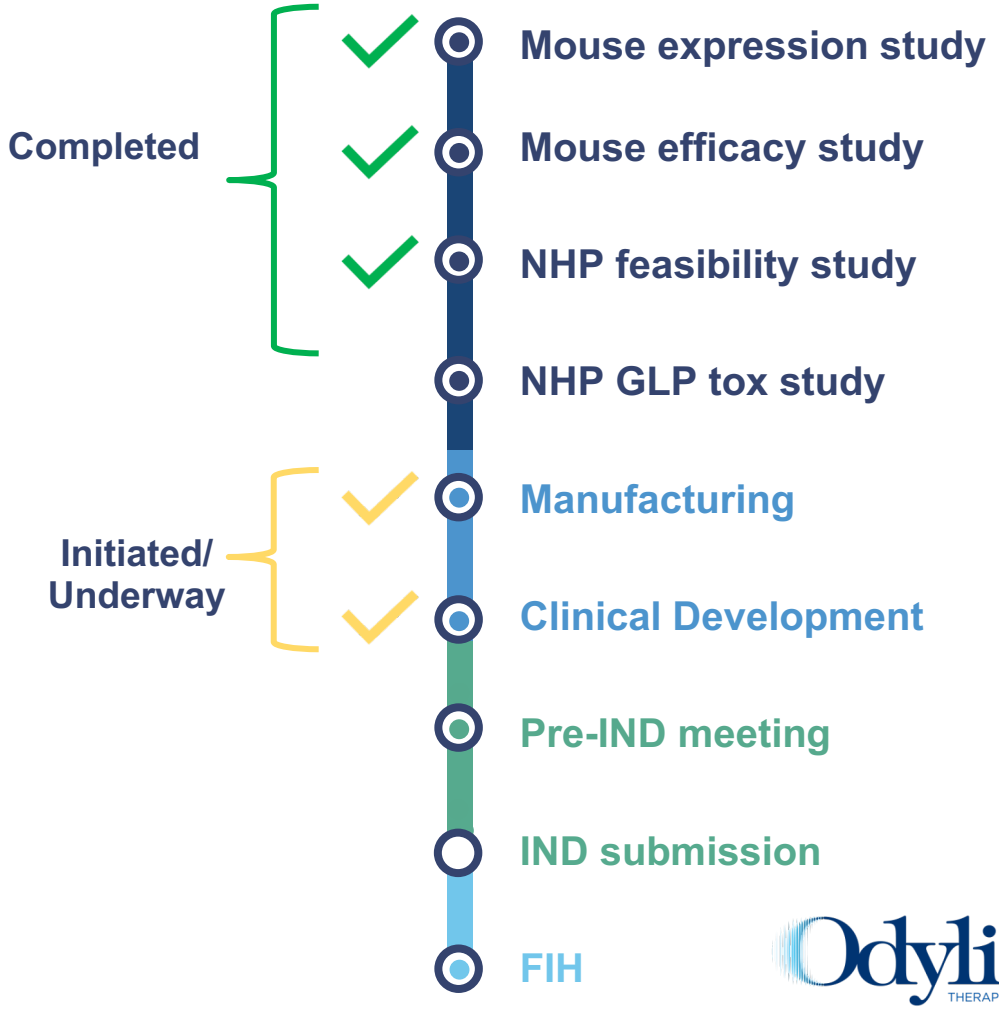
2019-2020

- Program in-licensed by PTC Therapeutics
- Biotech invests and formalizes proof-of-concept studies
- Builds upon prior dataset

2020- ongoing

- Program returned to Odylia
- Granted ODD & RPDD
- Initiating Tox and Manufacturing

DEVELOPMENT PATH



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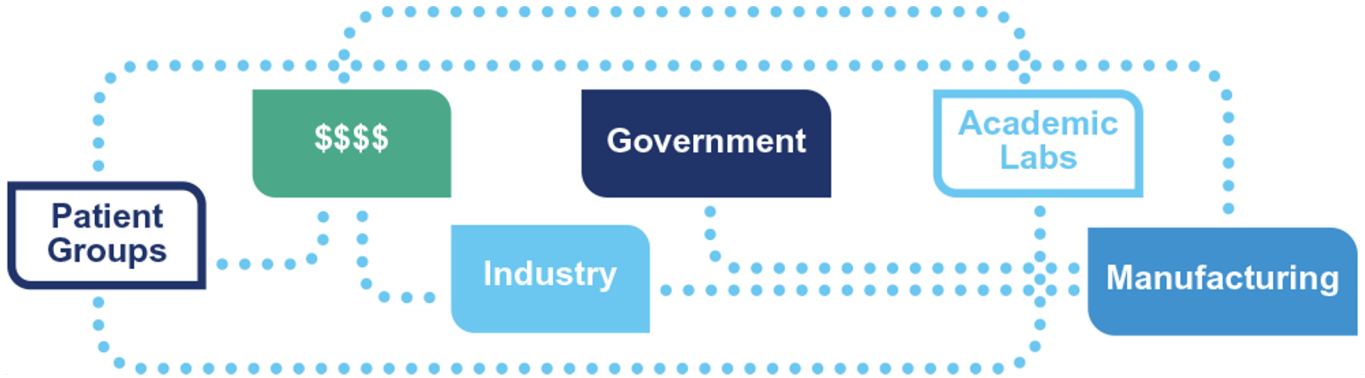
Partnering with Odylia

The Traditional Paradigm Doesn't Work for Many Rare Diseases

- Meaningful advancements toward treatments can be challenging and slow; paths to developing new drugs are not straightforward
- Development of treatments are driven by expected financial profit instead of potential human benefits so rare disease treatments are often deprioritized
- Thus, **promising treatments are not getting to people** who need them due to the small number of patients

The current commercial model does not work for most rare diseases



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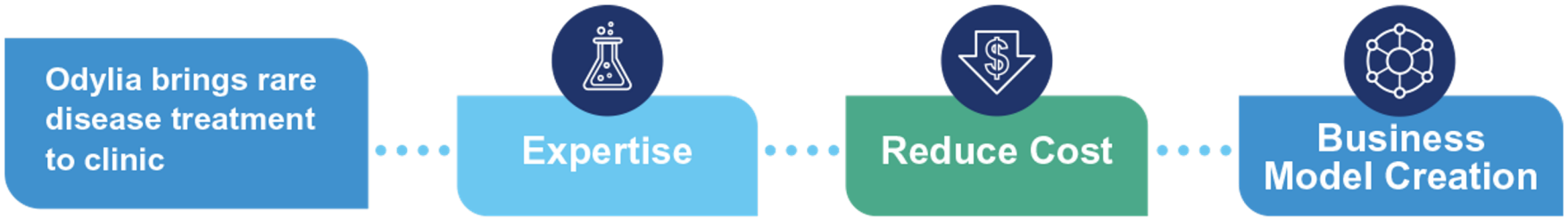
The Future with Odylia

Odylia navigates common obstacles for rare disease and mitigates risk

We do this through:

- a blend of science and business
- a patient-centric model, ensuring outcomes are focused on getting treatments to people, hand-in-hand with the patient community
- strategic research plans to lower costs, save time, and minimize risks
- creative collaboration with patient groups, academics labs, and drug manufacturers to advance treatments in timely, economical, and effective ways

Odylia remains nimble in order to create opportunities for treatments



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Odylia is Unique

Partnerships Drive Progress

- We are a **nonprofit** biotech with the sole goal of bringing treatments to patients as quickly and inexpensively as possible
  - most rare disease therapies are discovered in academic labs which often have different goals for success and do not have the know-how to develop drugs for clinical trial
- Odylia works directly with Patient Advocacy Groups who are the primary drivers of rare disease research
  - however, they often lack the scientific knowledge and bandwidth to determine a strategic, streamlined plan and are left to navigate complex drug development alone. Our expertise saves them time and money
- Each treatment program may require a different model to reach clinical trials
  - we adapt to meet the needs. Working with various researchers, labs, and manufacturers gives us options

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Supporting Odylia's Mission

- Odylia is seeking $625,000 to support annual operations to achieve our vision of bringing life changing treatment to people with genetic disease, regardless of prevalence or commercial interest

- Additionally, Odylia is looking support of the RPGRIP1 gene therapy program: $4.5 million over the next two years

We are also open to exploring alternative forms of partnership or philanthropy around the RPGRIP1 program

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