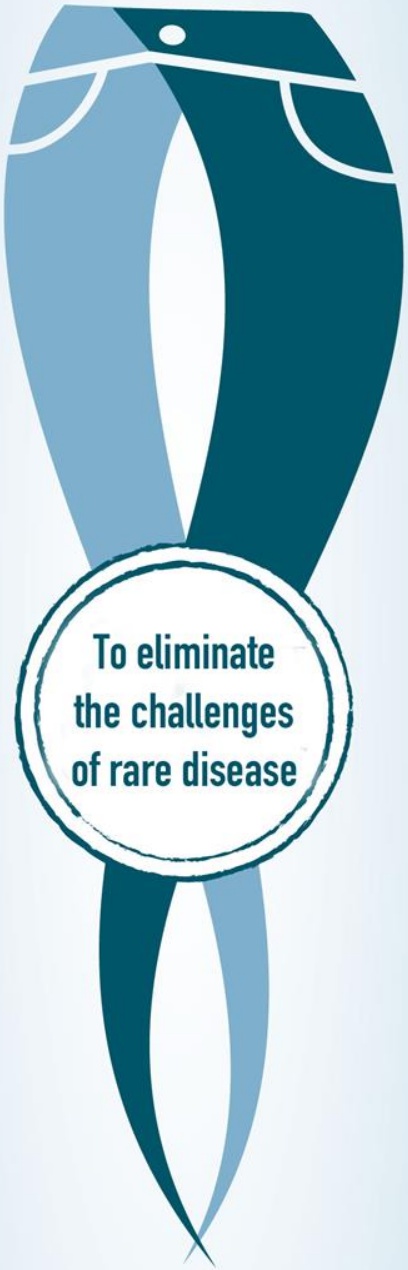

Global Genes™
Allies in Rare Disease

Understanding Gene Therapy

April 26, 2016

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RARE WEBINARS

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Meet Today's Panelists:

Panelists:

Karen Kozarsky, Ph.D
President, Vector BioPartners

Michelle Berg
Vice President, Patient Advocacy, Abeona Therapeutics

Alison Rockett Frase
President and Founder
Joshua Frase Foundation

Moderator:

Daniel Levine
Levine Media Group

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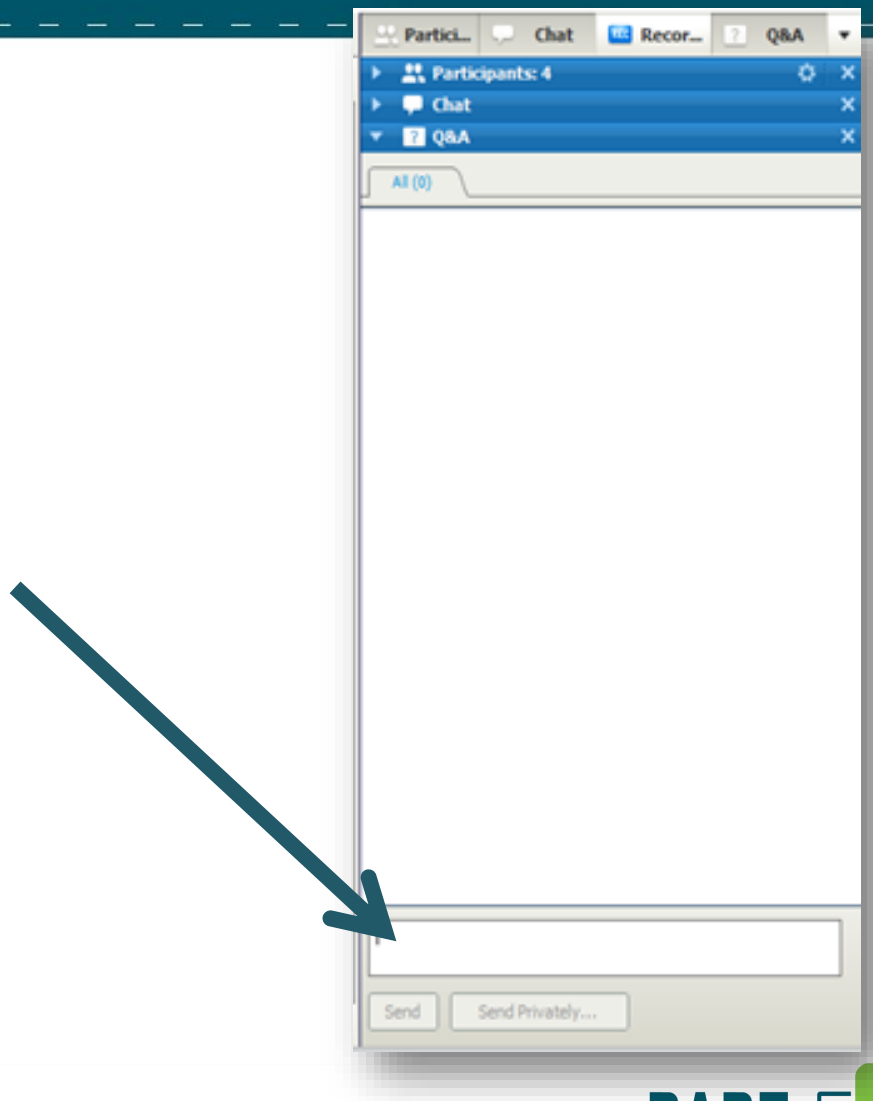
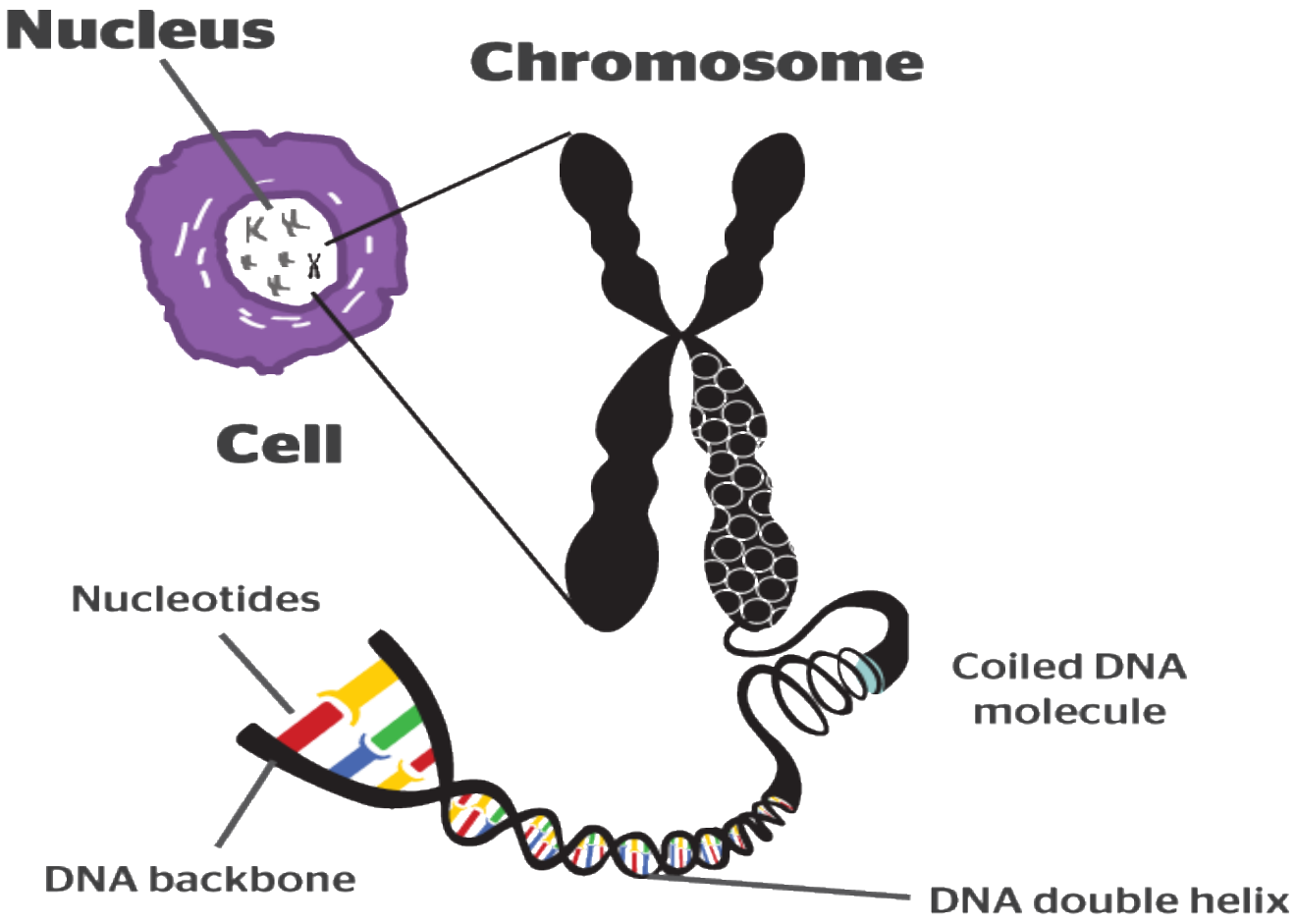


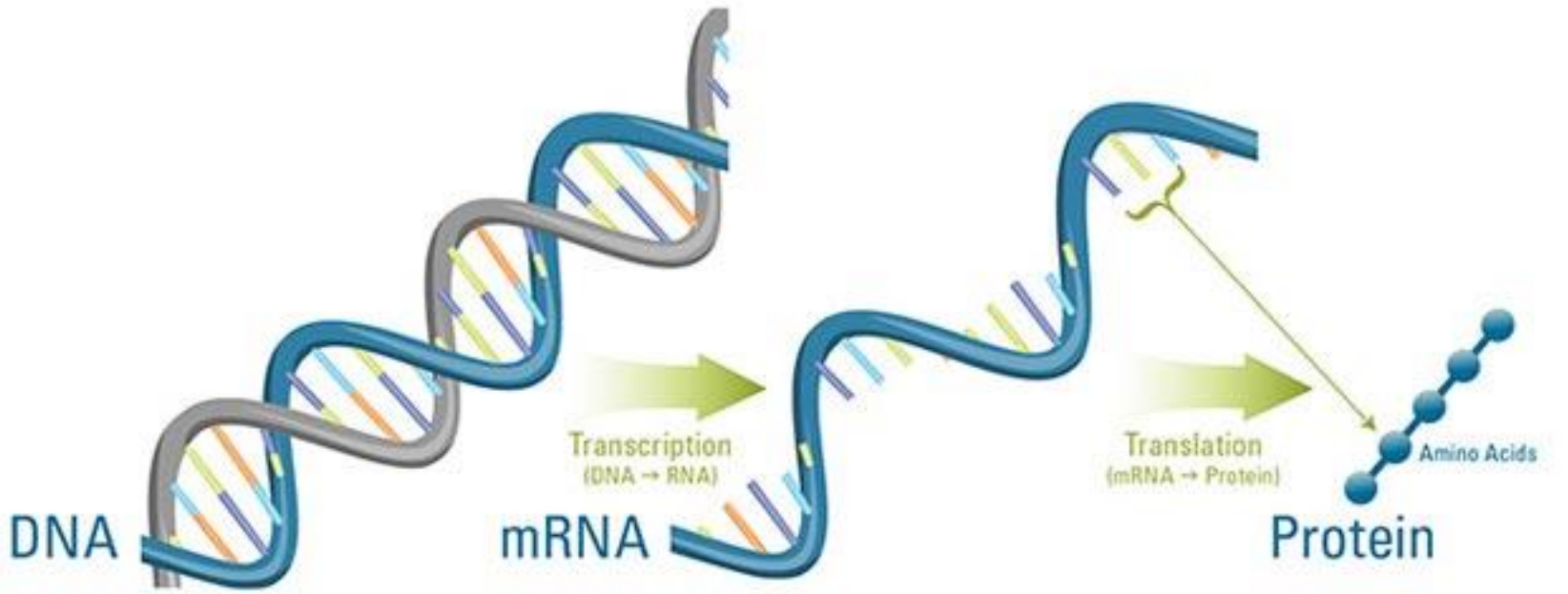
Karen Kozarsky, Ph.D

President, Vector BioPartners

Definitions

- Gene:
  - A segment of DNA found on a chromosome that codes for a particular protein
  - Humans have approximately 100,000 genes that act as a blueprint for making specific enzymes or other proteins for virtually every biomedical reaction and structure in the body


Nucleus
Chromosome
Cell
Nucleotides
Coiled DNA molecule
DNA backbone
DNA double helix


DNA
Transcription
(DNA → RNA)
mRNA
Translation
(mRNA → Protein)
Amino Acids
Protein

Definitions

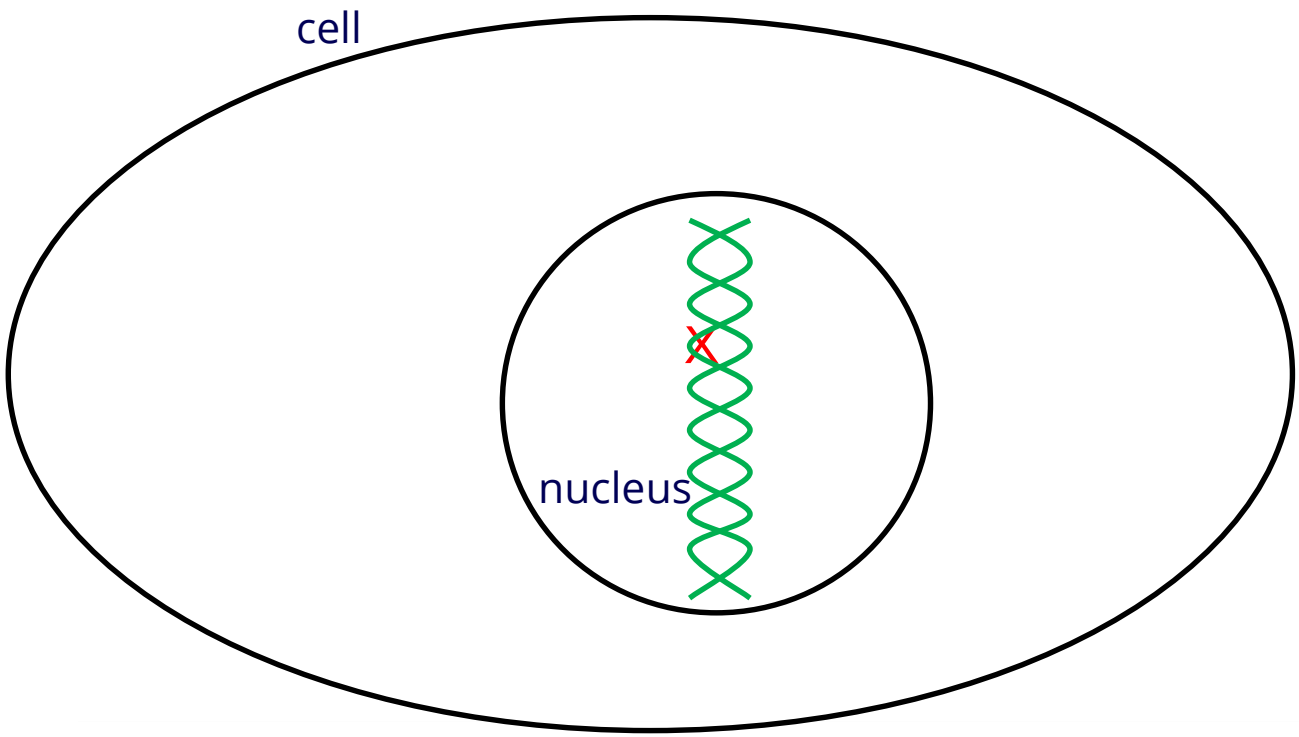
- Gene Therapy:
  - The treatment of disease by either replacing damaged or abnormal genes with normal ones, or by providing new genetic instructions to help fight disease.
  - Therapeutic genes are transferred into the patient either through a weakened virus, a non-viral vector, or through direct delivery of so-called "naked" DNA

Types of Gene Therapy

- Gene replacement:
  - Replacement/addition of a normal gene, where a gene is defective or missing
    - Example: Hemophilia B – Replace clotting Factor IX
- Gene addition:
  - Expressing a therapeutic gene at the site needed
  - Most used in more complex disorders
    - Example: Wet age-related macular degeneration (wet AMD)
- Modulation of gene expression: reducing effects of a deleterious gene product
  - Silencing gene expression (RNAi)
  - Example: autosomal dominant retinitis pigmentosa

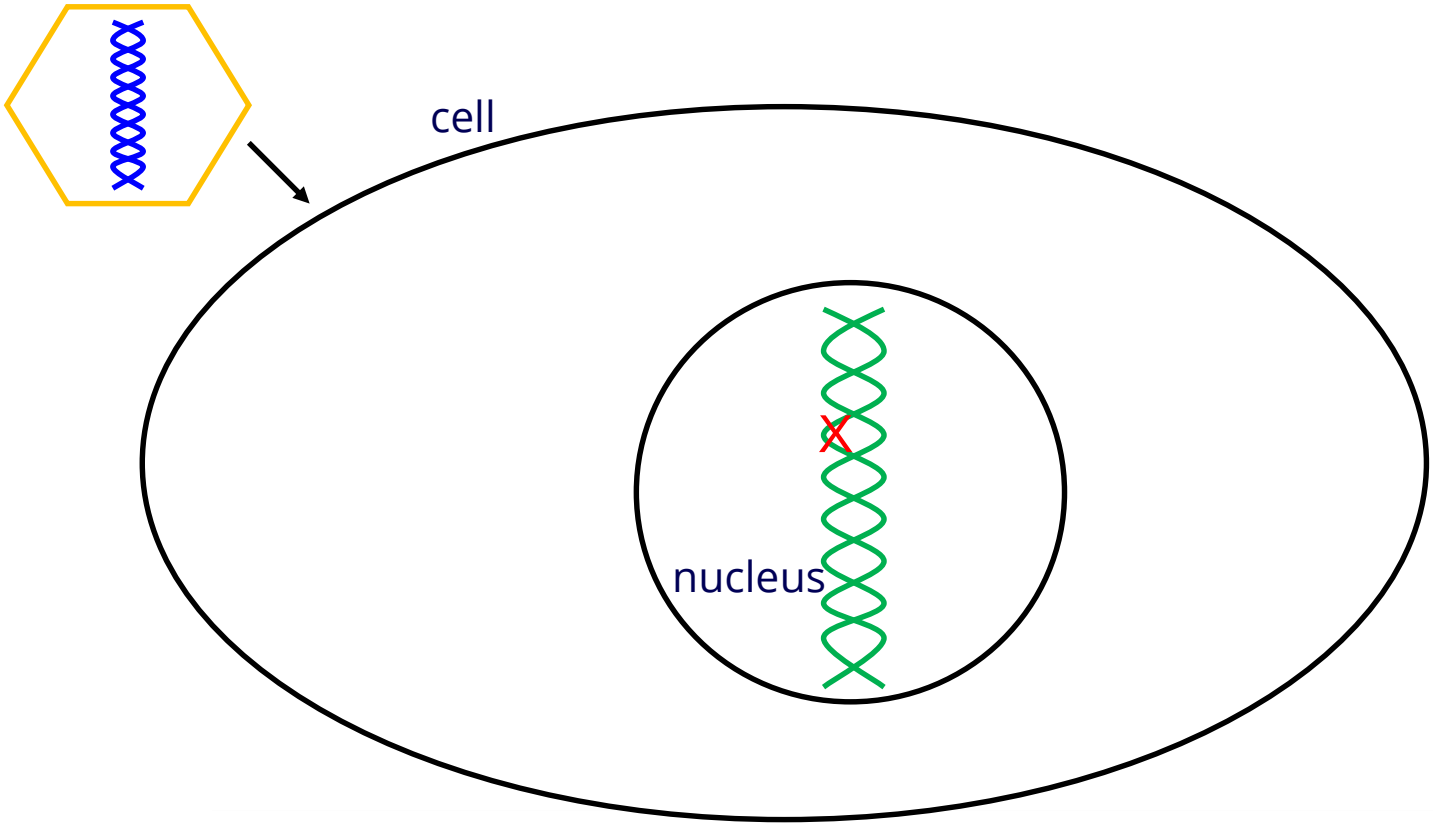
Gene Therapy

Problem: How to deliver correct copy of gene to cell?
DNA is large, highly charged



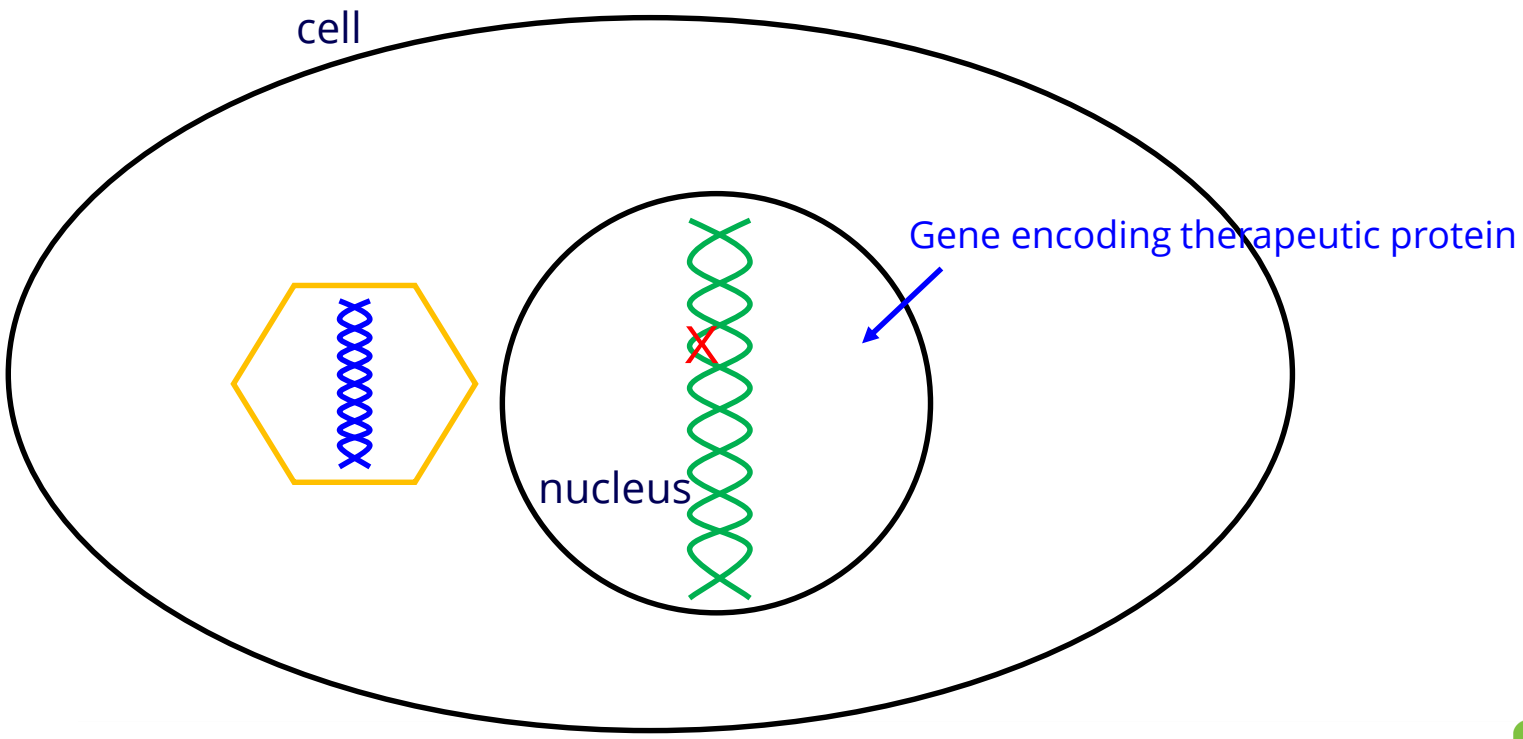
Gene Therapy

Solution: Use a virus, which evolved to deliver its genes efficiently to cells
Disable virus so it delivers genes but does not replicate



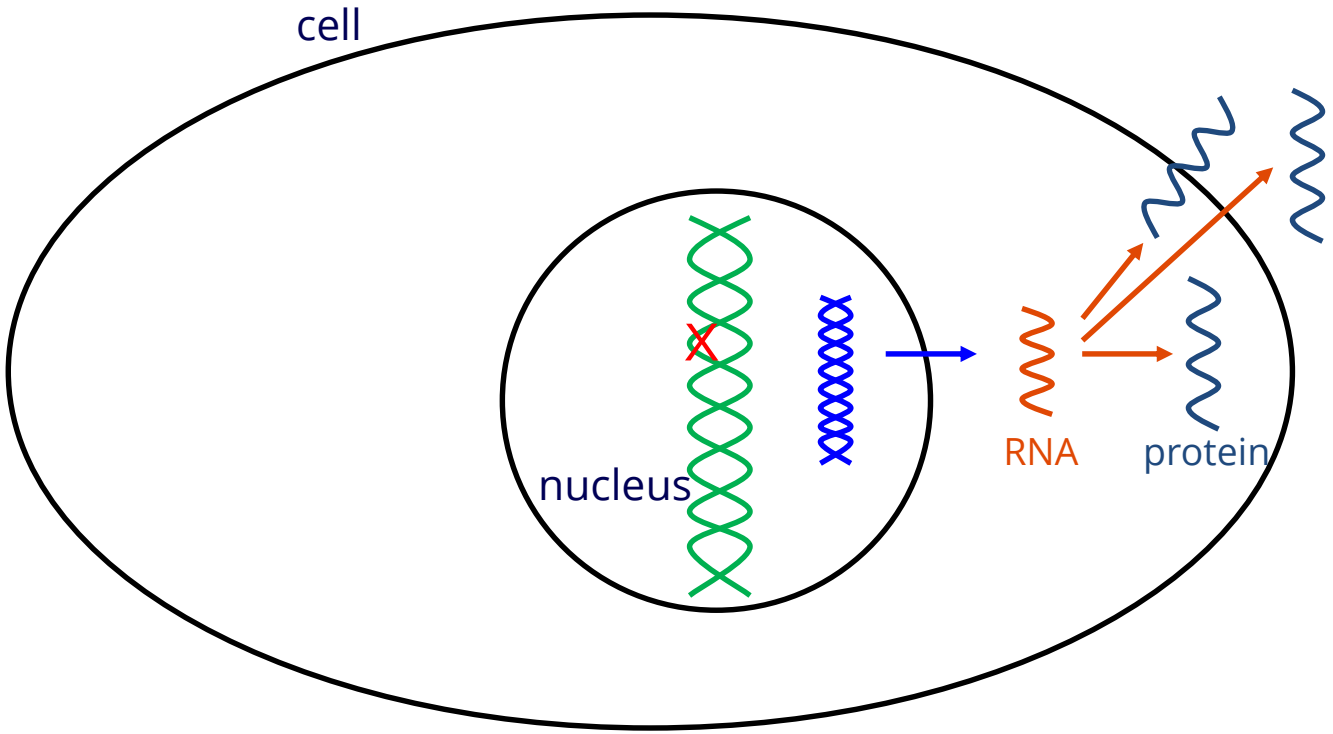
Gene Therapy

Virus uncoats, genetic material enters nucleus



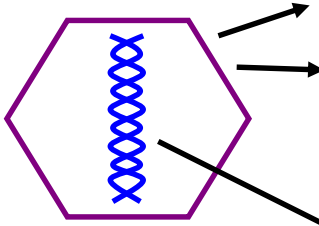
Gene Therapy

The DNA delivered contains instructions to make therapeutic protein



Need to select appropriate recombinant virus and appropriate transgene for each disease

Vector Design

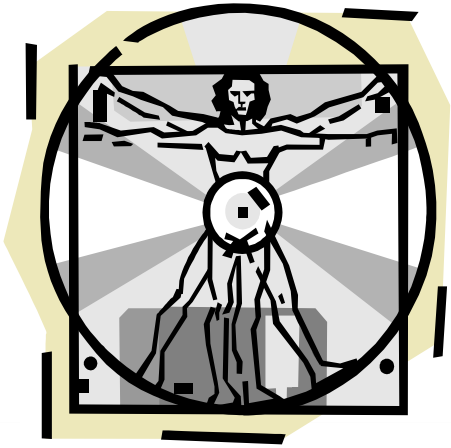


Virus selection based on cell types/tissue for delivery

Variations in virus coat can improve efficiency

What gene to deliver?

Where to deliver the virus?

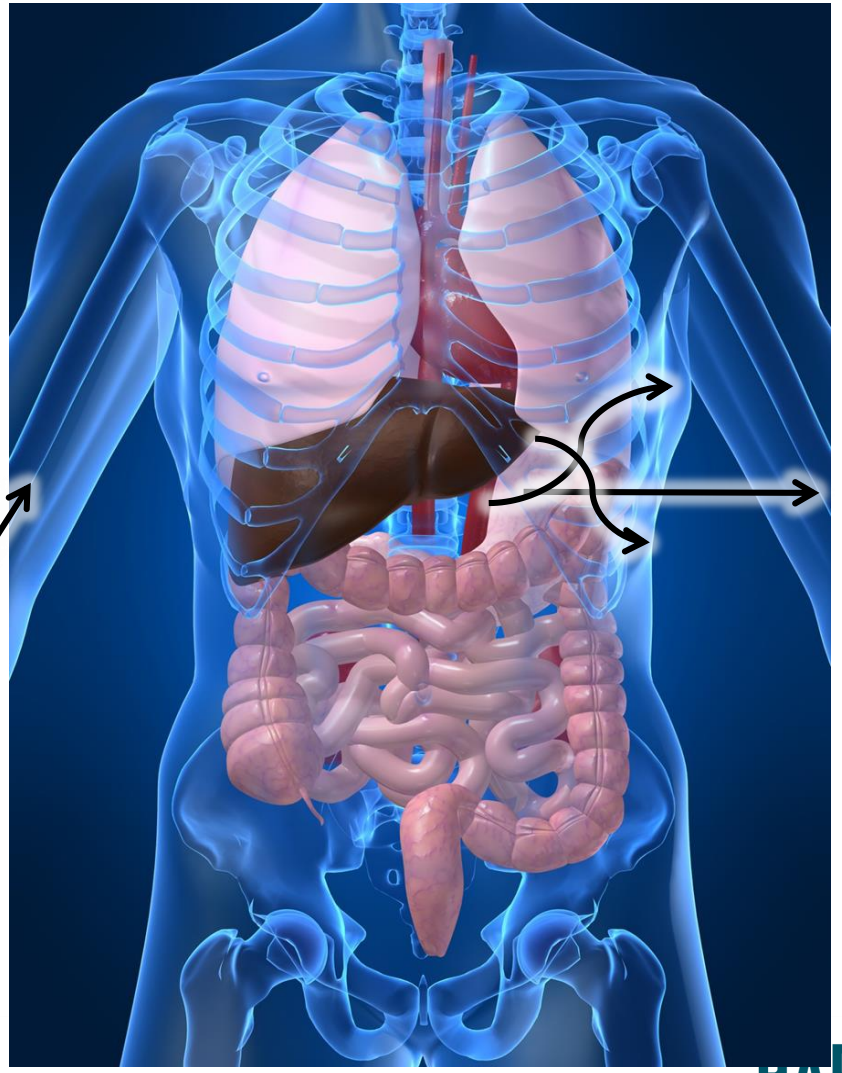


Example: Hemophilia B

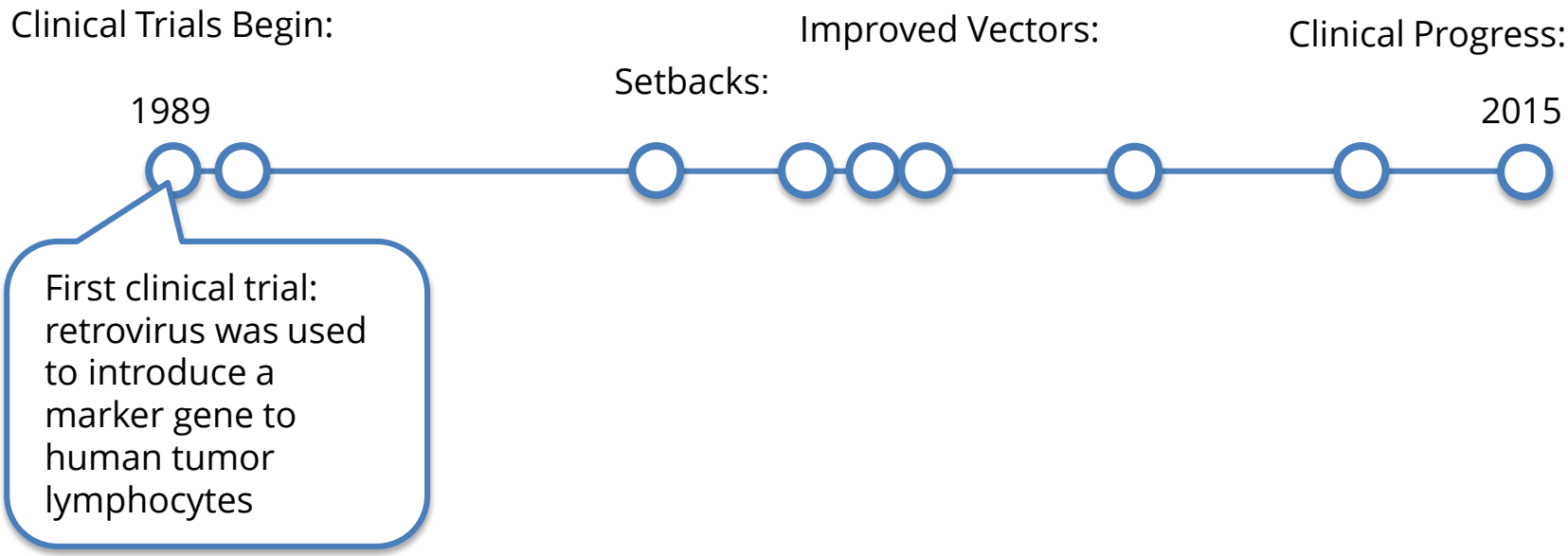
- Factor IX protein is part of the clotting cascade
- Patients with hemophilia B have mutations in the Factor IX gene
  - Results in lowered ability of blood to clot
  - Bleed heavily after injury
  - Severely affected patients prone to spontaneous bleeds

Treatment being tested in the clinic:

- Intravenous injection of an AAV vector carrying a normal copy of the Factor IX gene
- Liver synthesizes, secretes FIX



Gene Therapy Milestones in Rare



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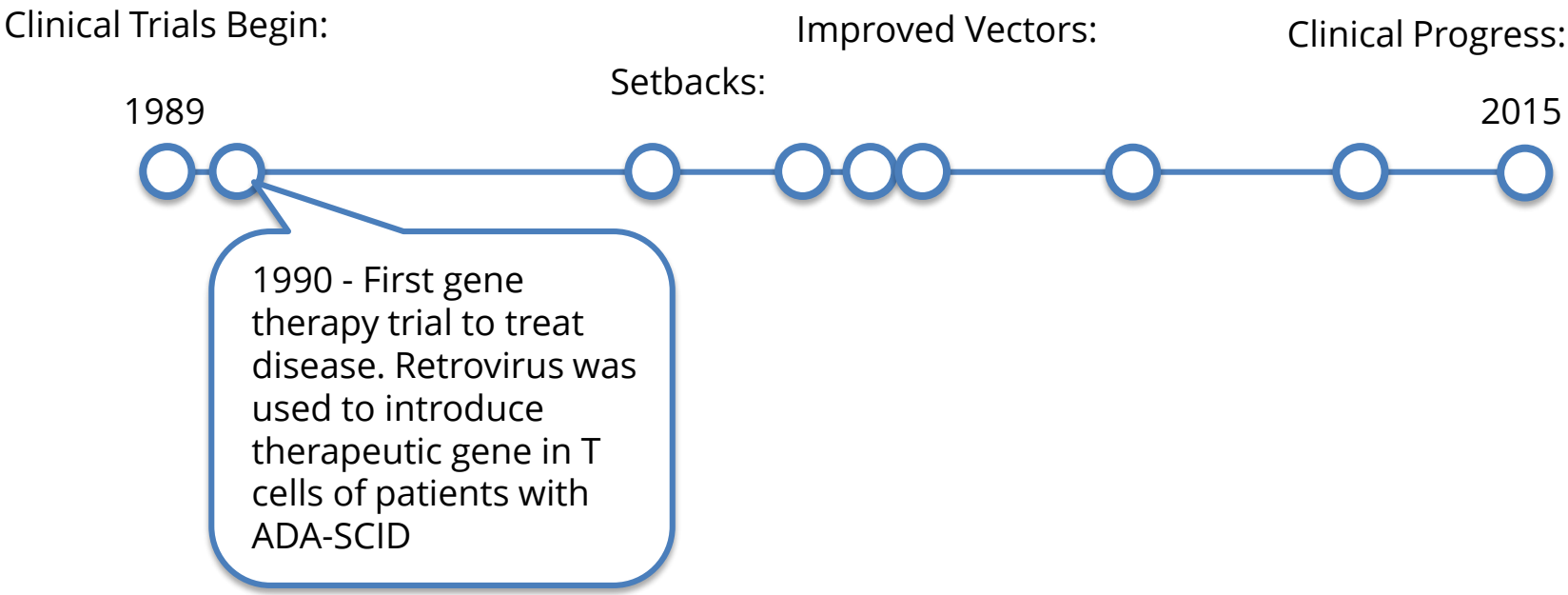

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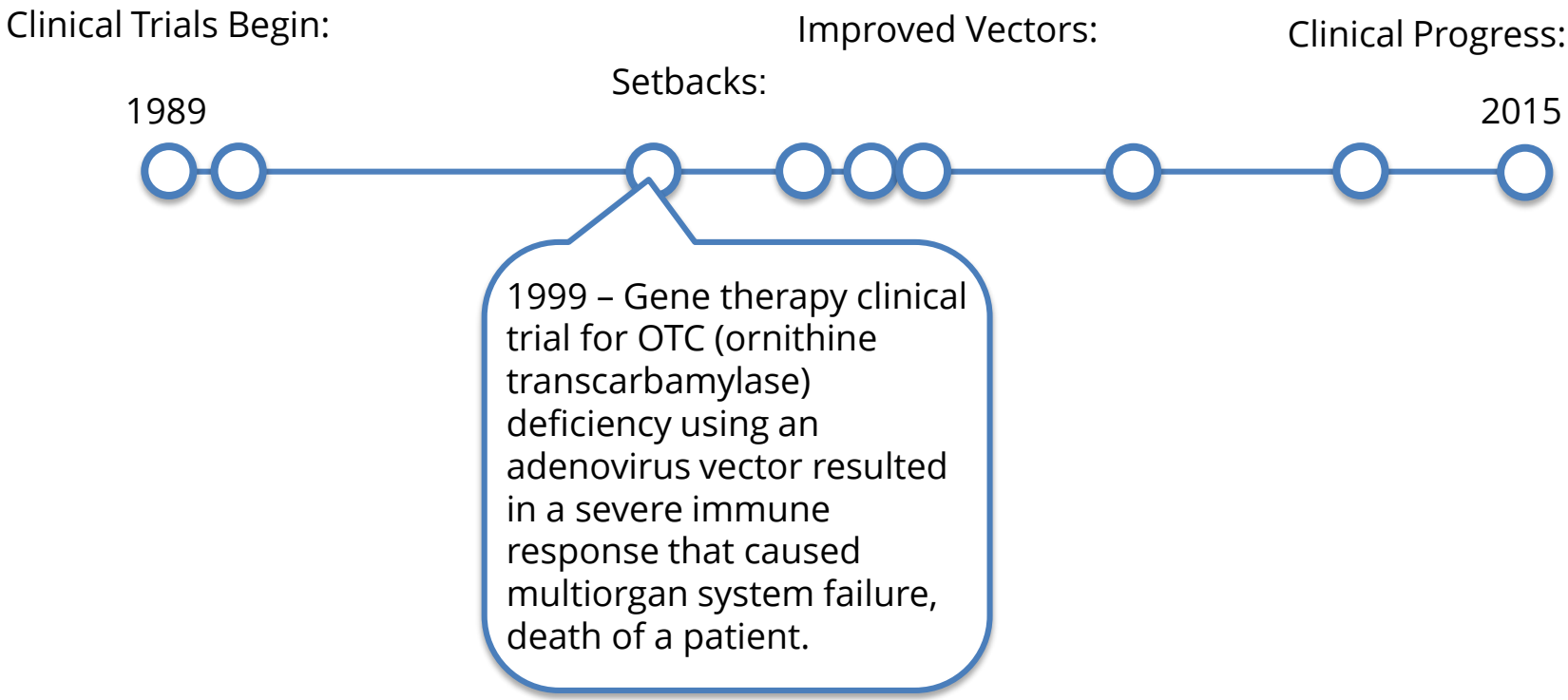

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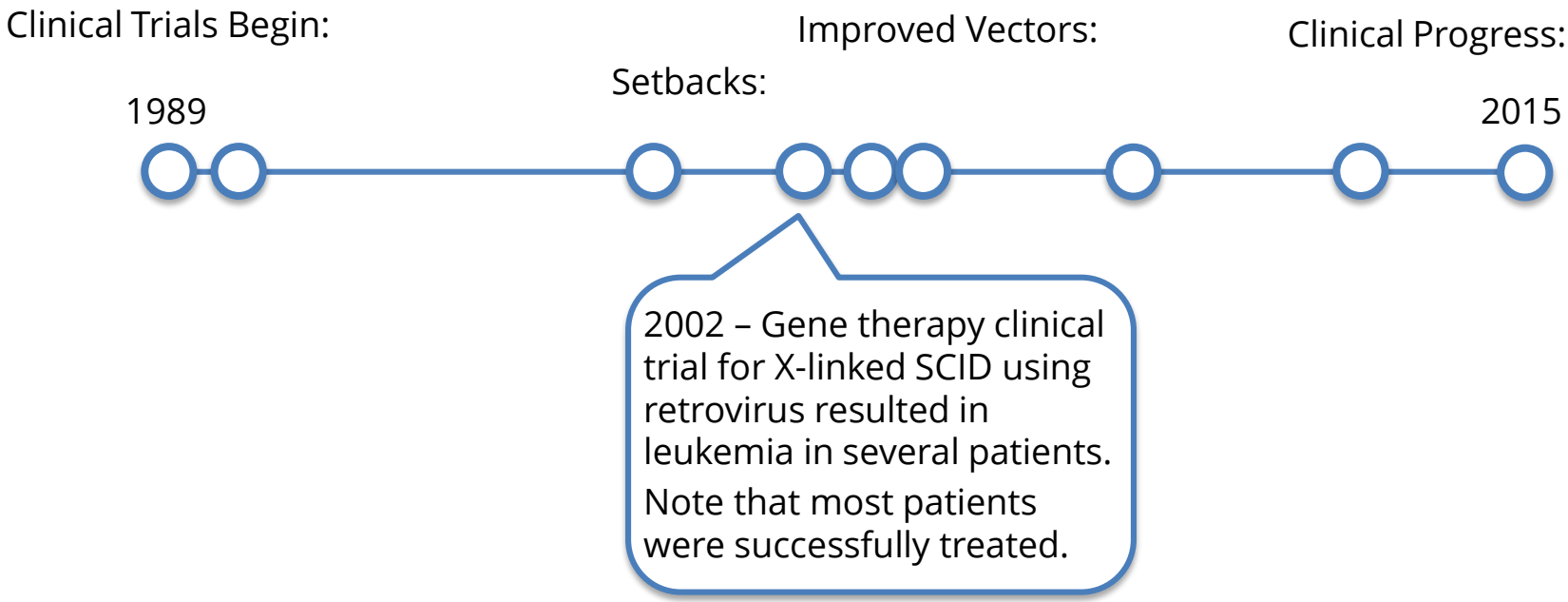

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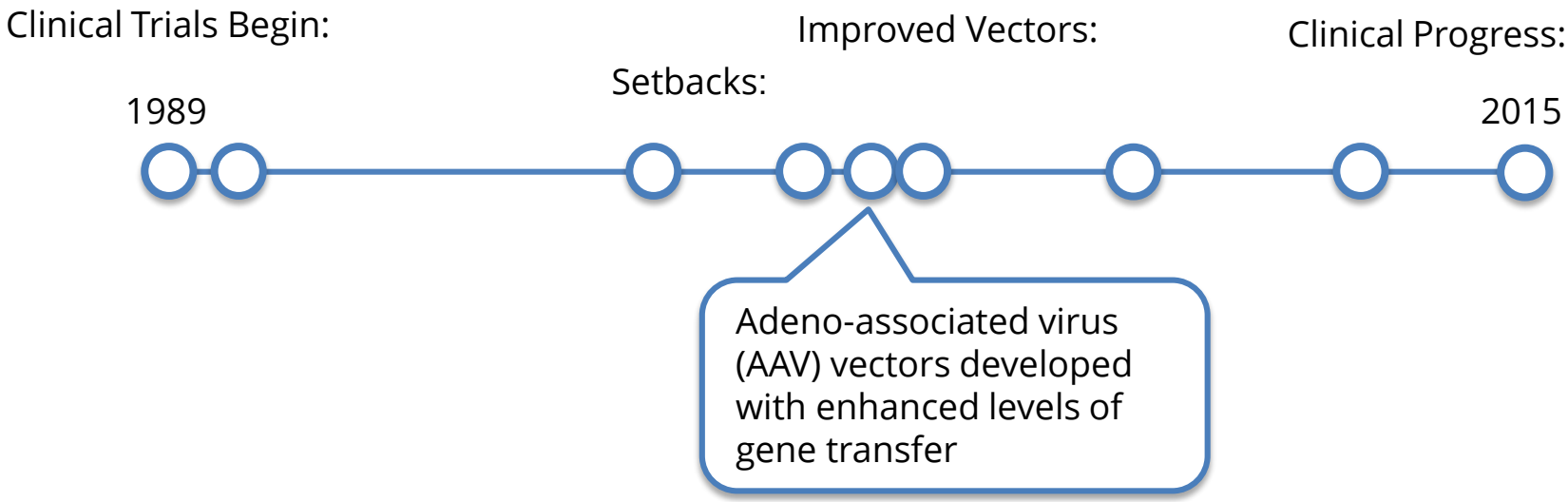

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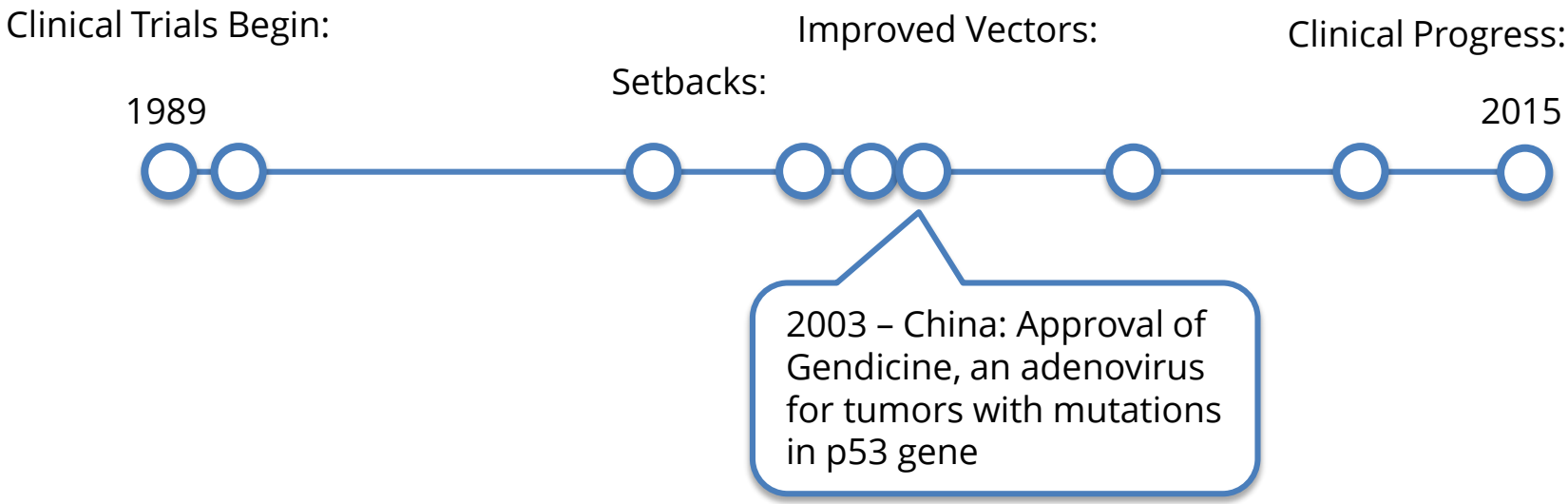

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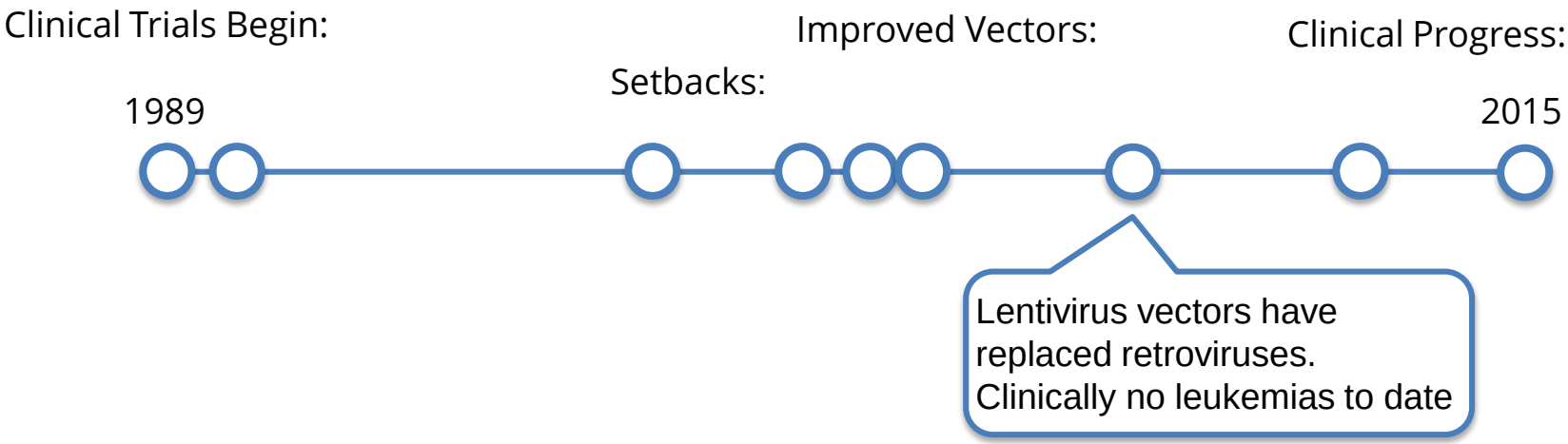

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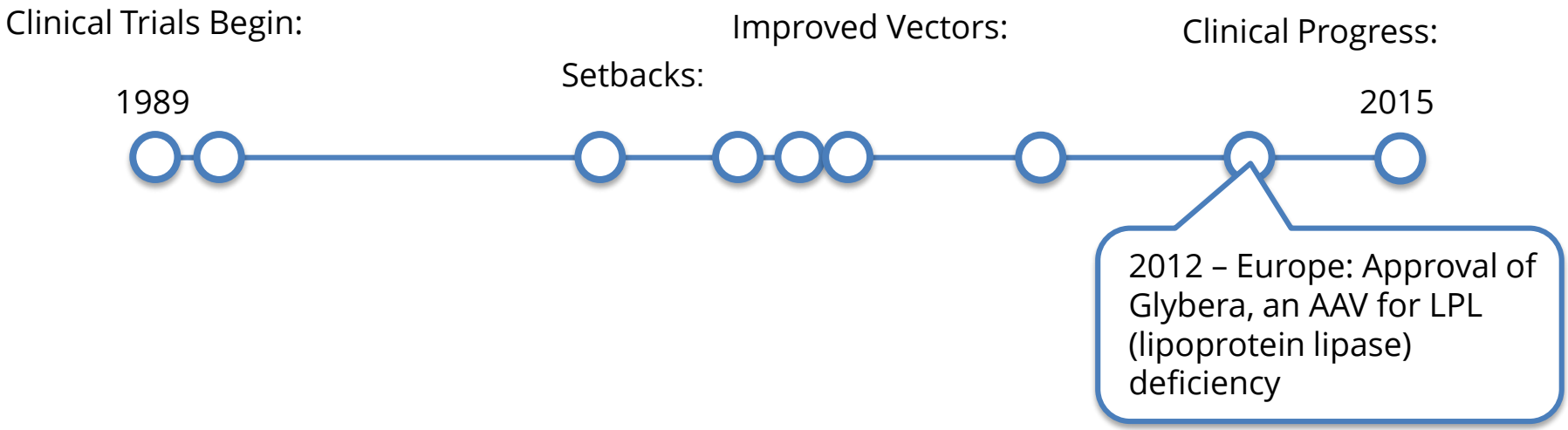

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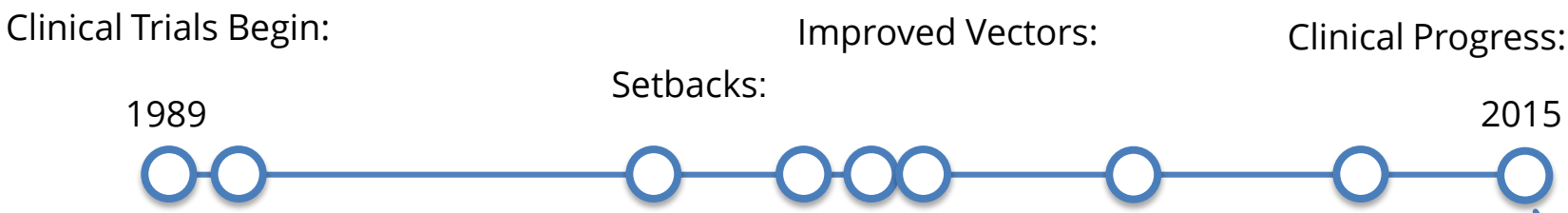


Over 2200 gene therapy clinical trials have been initiated WW

Includes ~100 late-phase/registrational trials

Michelle Berg

Vice President, Patient Advocacy, Abeona Therapeutics

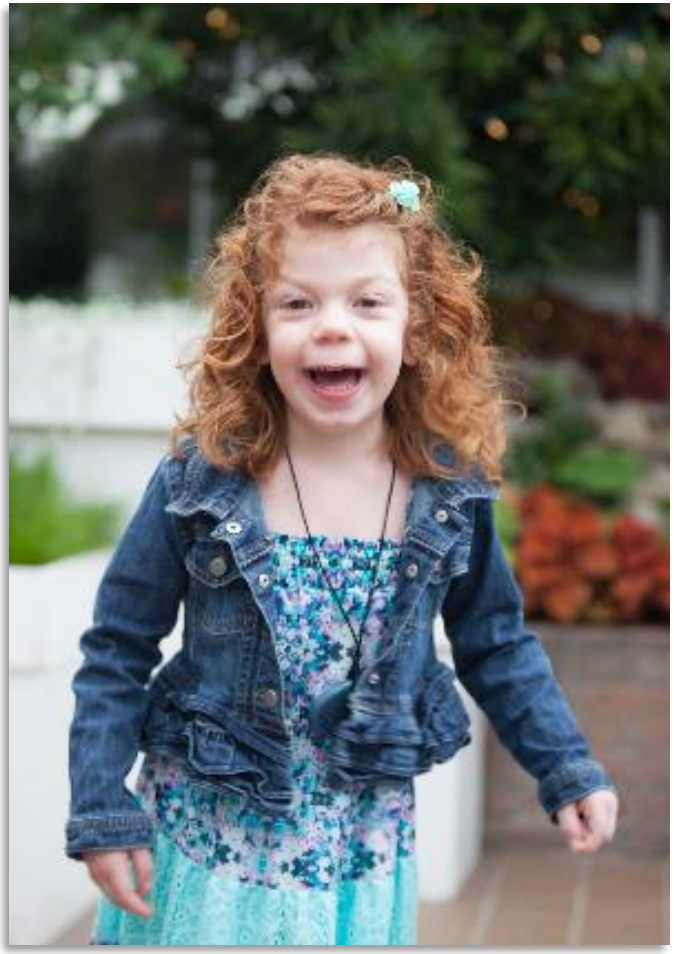
Safe Harbor Statement

This presentation contains certain statements that may be forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, including statements relating to the product portfolio and pipeline and clinical programs of the company, the market opportunities for the all of the company's products and product candidates, and the company's goals and objectives. These statements are subject to numerous risks and uncertainties, including but not limited to the risks detailed in the Company's Annual Report on Form 10-K for the year ended December 31, 2014, and other reports filed by the company with the Securities and Exchange Commission.

This presentation does not constitute an offer or invitation for the sale or purchase of securities or to engage in any other transaction with Abeona Therapeutics or its affiliates. The information in this presentation is not targeted at the residents of any particular country or jurisdiction and is not intended for distribution to, or use by, any person in any jurisdiction or country where such distribution or use would be contrary to local laws or regulations. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this presentation or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise.

The Name

- **Pronunciation:** *ā-bē-ō-nuh*
- **Origin:** Roman Goddess said to be the protector of children as they start out on their journey.
- **Mission:** Abeona Therapeutics develops and delivers gene therapy and plasma-based products for severe and life- threatening rare diseases.



Abeona Therapeutics History

- Founded in March 2013 out of Cleveland, OH
- Licensed technology from Nationwide Children's Hospital
- Lead products – gene therapy for MPS IIIA and IIIB
- Received the FDA Orphan Drug Designations for MPS IIIA and IIIB clinical programs
- Received Pediatric Rare Disease Designations
- Raised $6M from over dozen international foundations

Our Pillars of Support

STOPSANFILIPPO FUNDACIÓN — Spain

fundación SANFILIPPO B — Spain

TEAM Sanfilippo FOUNDATION — USA

FONDATION SANFILIPPO Suisse — Switzerland

ABBY GRACE FOUNDATION Cure Sanfilippo — USA

The Children's Medical Research Foundation, Inc.® A Cure for Kirby — USA

Ben's Dream The Sanfilippo Research Foundation — USA

The Sanfilippo Children's Research Foundation — Canada

National MPS Society Support for Families. Research for a Cure. — USA

USA

REDSANFILIPPO FUNDACIÓN — Mexico

Sanfilippo Children's Foundation Funding research, finding gems — Australia

From Private to Public

Abeona therapeutics

↓

PlasmaTech Biopharmaceuticals

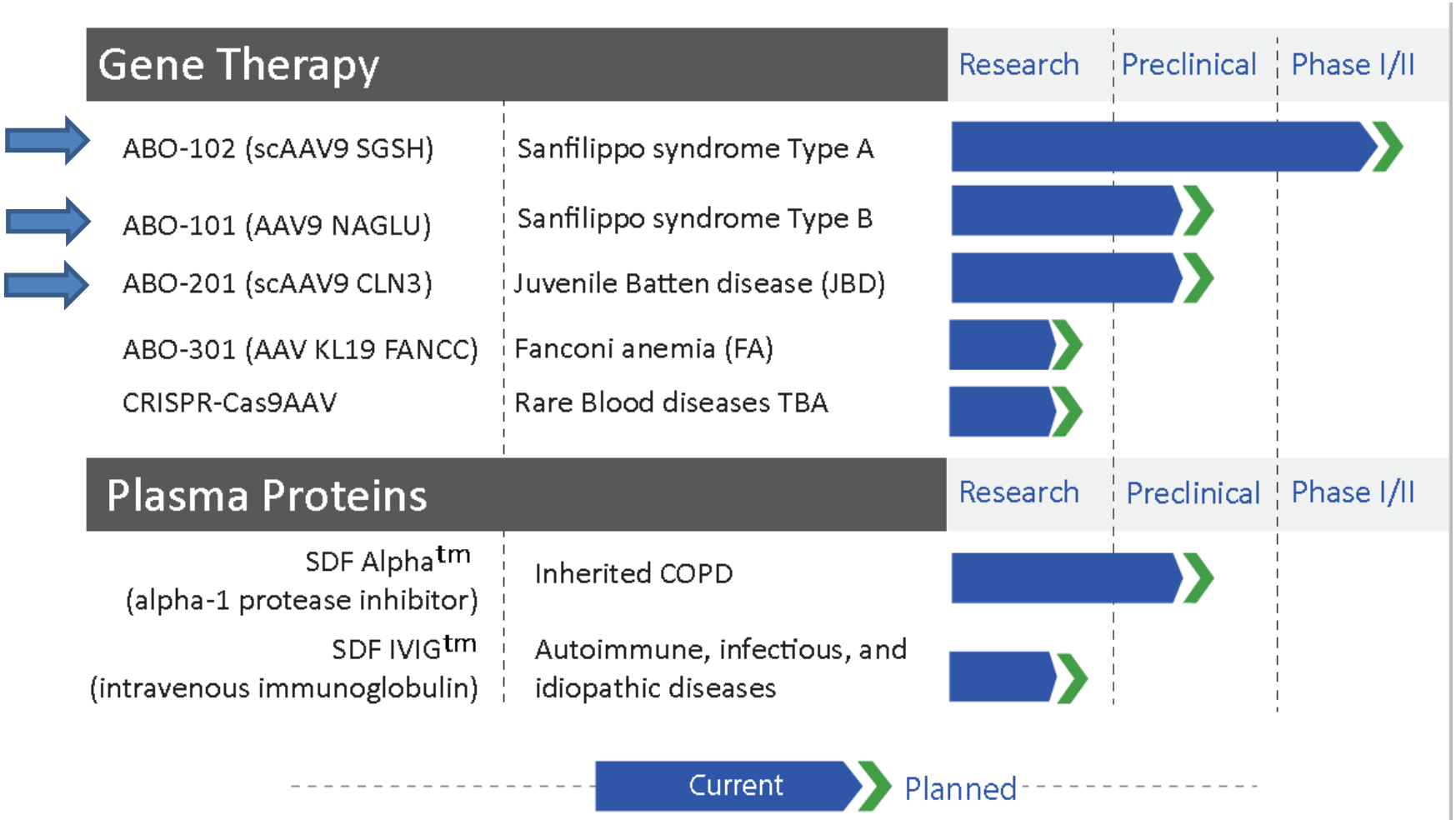
↓

Abeona THERAPEUTICS

Working together to find a cure.

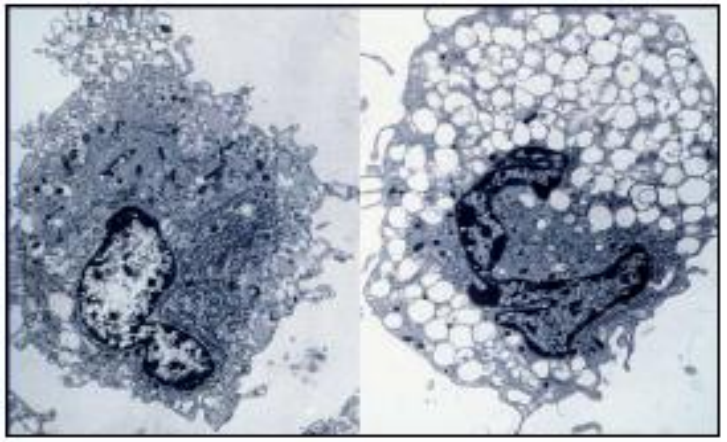
June 22nd, 2015: My first day!

Our Potential Therapies



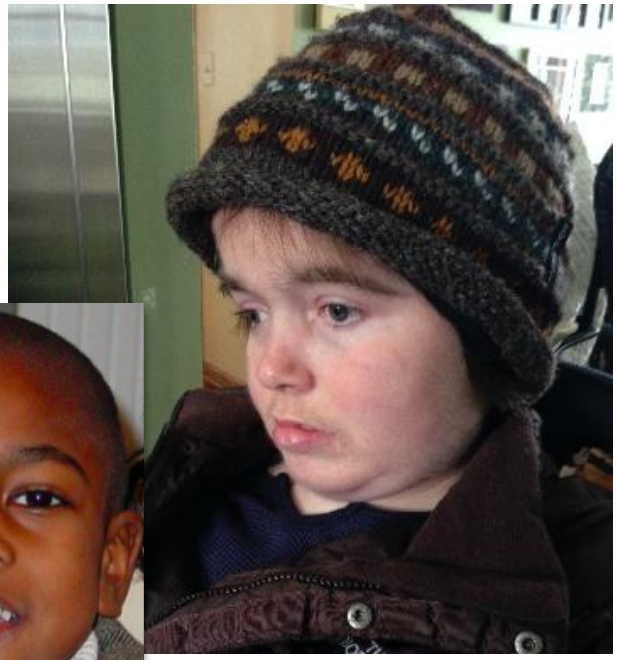
Developing Gene Therapy for Lysosomal Storage Diseases (LSD)

- Over 60 types
- Caused by an inherited deficiency in enzyme
- Buildup of materials in cells
- Progressive, severe neurological and muscular disorders
- Few current treatments approved or in development



Normal Cell Cell with LSD

About Sanfilippo Syndrome



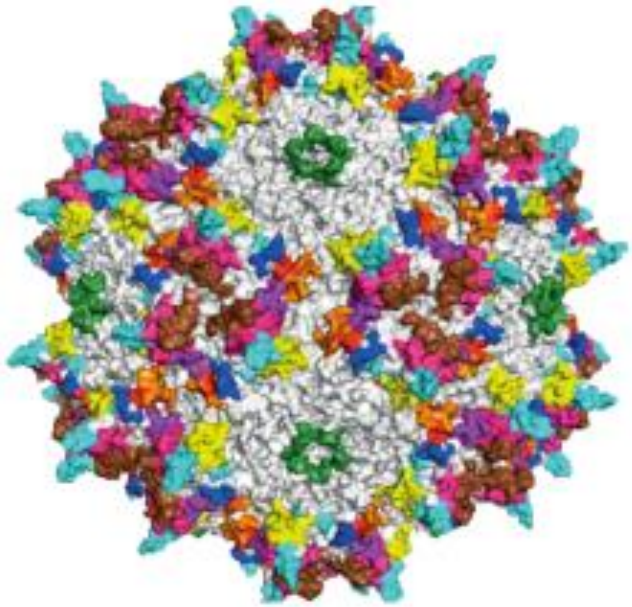
- Mucopolysaccharidosis (MPS) Type III
- Estimated incidence is 1 in 70,000
- MPS IIIA (SGSH) and IIIB(NAGLU)
- Accumulation of glycosaminoglycans (GAGs) or Sugars
- Age of onset, 2-6 years old
- Behavioral and sleep disturbances, progressive, severe deterioration in CNS and body, fatal

About Juvenile Batten Disease

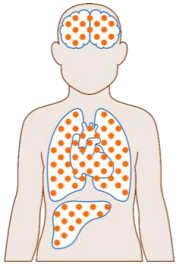
- Juvenile Neronal Ceroid Lipofuscinosis (JNCL), CLN3 gene
- Estimated incidence is 1:100,000
- Accumulation of lipopigments and proteins
- Age of onset is between 5-10
- Blindness, seizures, behavioral and sleep issues, progressive loss of motor and cognitive abilities, fatal

Selecting a Delivery Vehicle

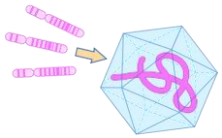
- Adeno-Associated Virus (AAV)
- Unable to replicate on its own
- Non-pathogenic
- Long-term expression
- Able to target specific tissues
- Safety demonstrated in clinical trial
- Commercial use: Glybera



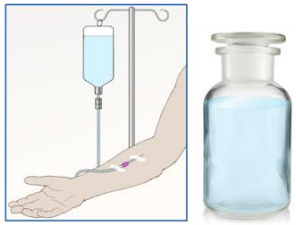
Delivering to the Whole Body



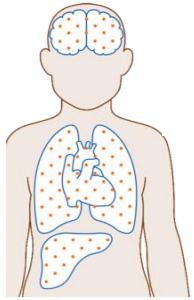
LSD affect all organs



AAV9 virus



Intravenous injection

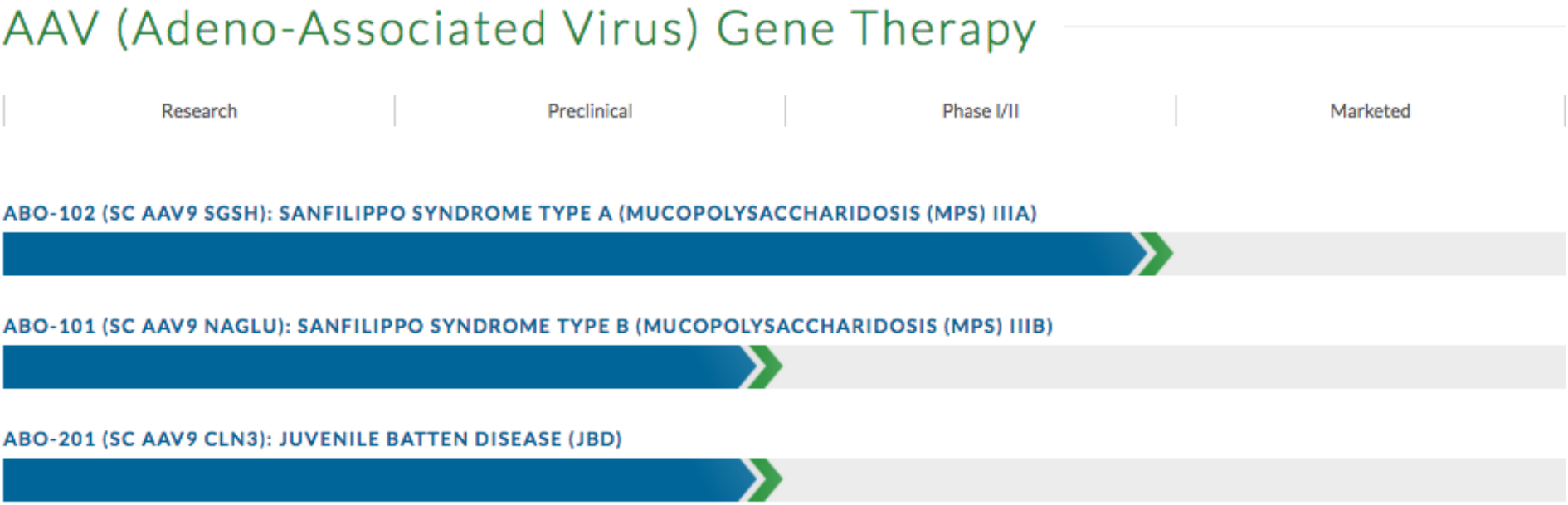


Whole-body correction

- Delivery and expression throughout the body, including CNS
- Clearance from cells in under 2 weeks
- Correction of neuromuscular and cognitive functions
- 100% improved survival

Demonstrated in mouse models of clinically relevant age

Important next steps



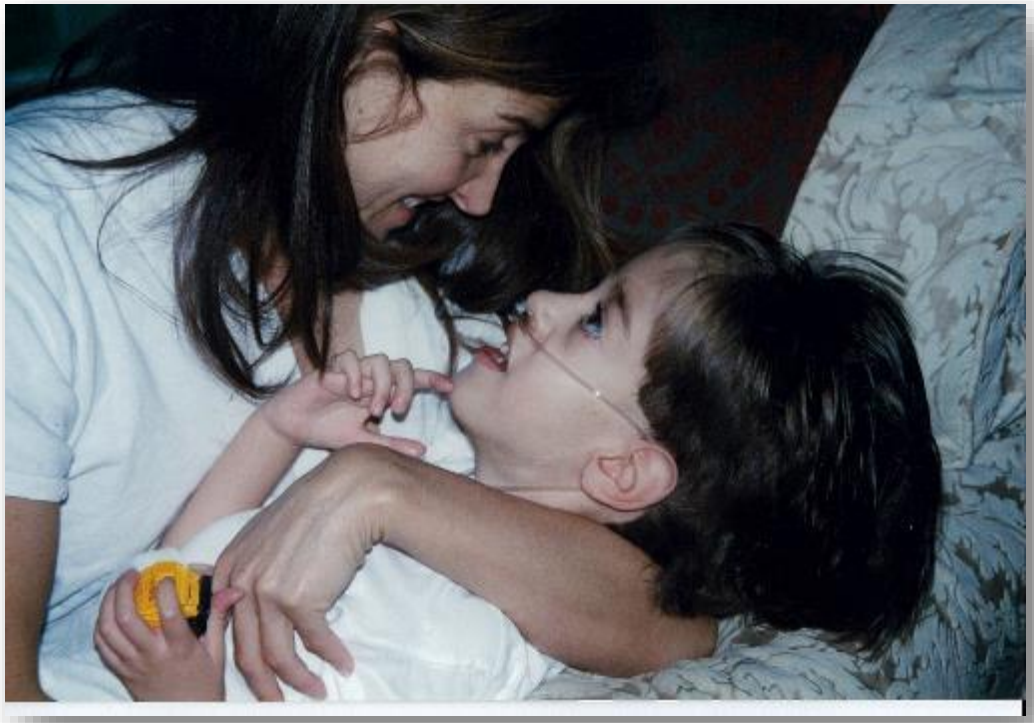
Academic Partners of Excellence

ABO-101 (AAV9 NAGLU) and **ABO-102** (scAAV9 SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB)	Nationwide Children's — When your child needs a hospital, everything matters.™
ABO-201 (scAAV9 CLN3) adeno-associated virus (AAV)-based gene therapy for juvenile Batten disease (JBD)	UNMC — BREAKTHROUGHS FOR LIFE.® The Ohio State University Medical Center
ABO-301 (AAV FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy program for rare blood diseases.	Stanford School of Medicine — Stanford University Medical Center University of Minnesota — Driven to Discover℠

A Foundation of Collaboration

Alison Rockett Frase

President and Founder, Joshua Frase Foundation



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Allies in Rare Disease

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Rock Star Team

Global Genes™
Allies in Rare Disease

Understanding Gene Therapy

RARE WEBINARS

Understanding Gene Therapy

Questions and Answers

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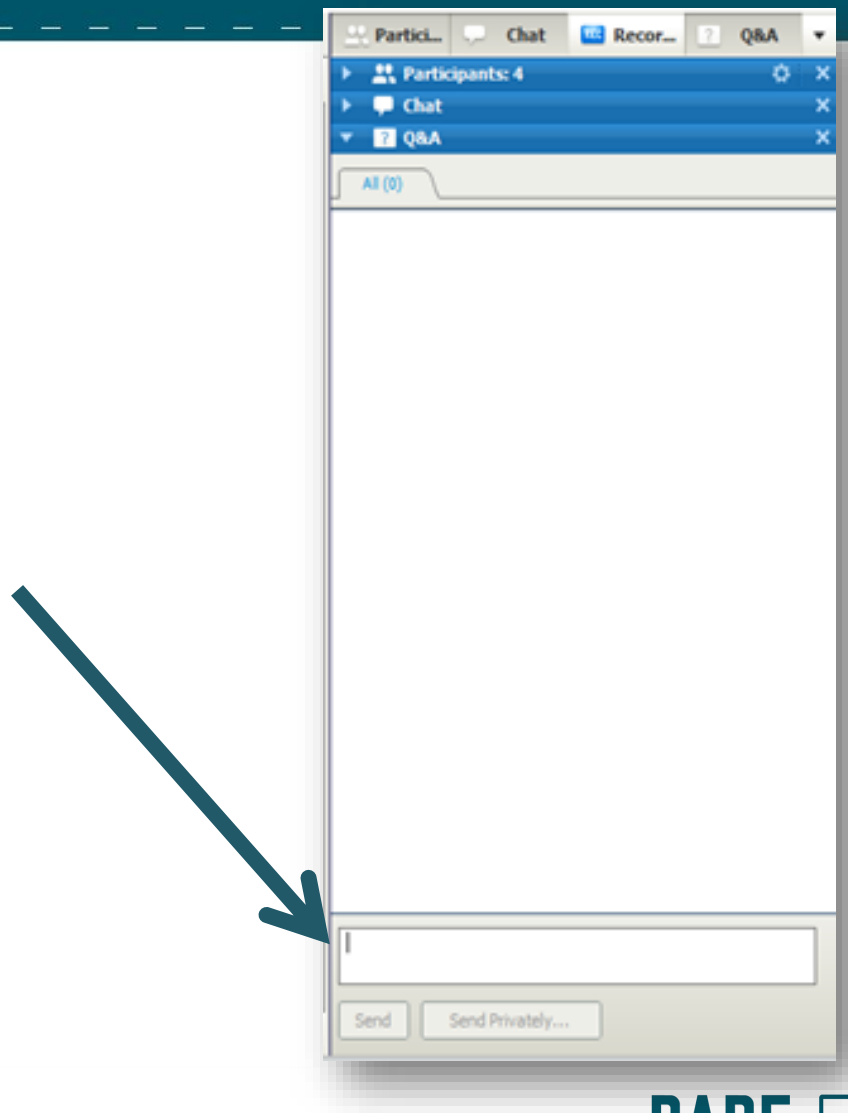

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Joshua Frase Foundation

Moderator:

Daniel Levine
Levine Media Group

Be sure to join us for our next webinar

Strategies for Effective Fundraising

On June 21, 2016

Registration opening soon

Look out for our toolkit

Understanding Gene Therapy

Coming at the end of May

Rare Patient Advocate Summit 2016 is coming

Registration opening soon