



Rare Disease Advocacy in Action

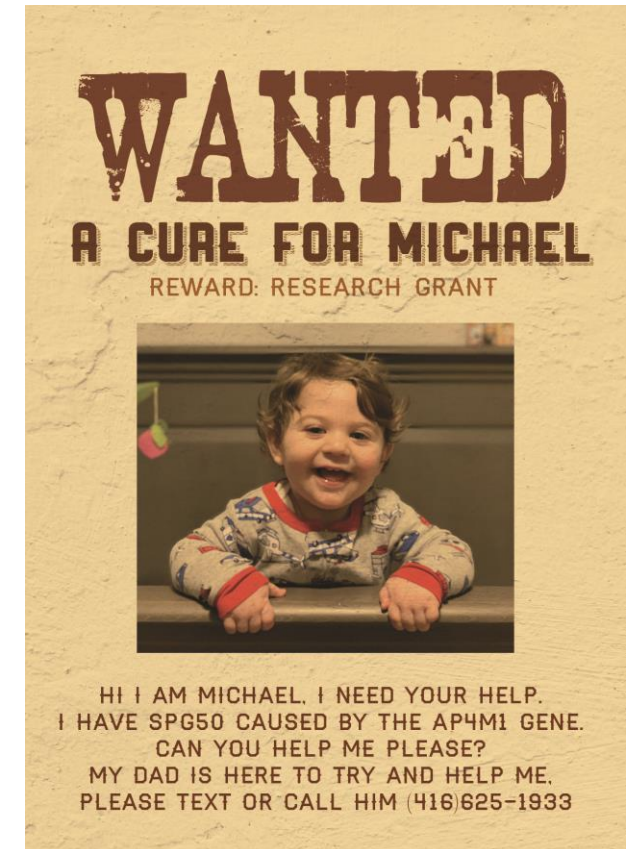
Rachel Thomas, RN
Senior Project Manager

Terry's Journey

April 2019

Spastic Paraplegia Type 50 (SPG50)

- Low Muscle Tone
- Microcephaly
- Most patients have lower body paralysis from spasticity by age 10
- Quadriplegic by second decade of life



Terry's Journey

March 2022

Gene Therapy AAV9/AP4M1

- Preclinical studies
- Health Canada approval
- FDA IND



Dr. Steven Gray

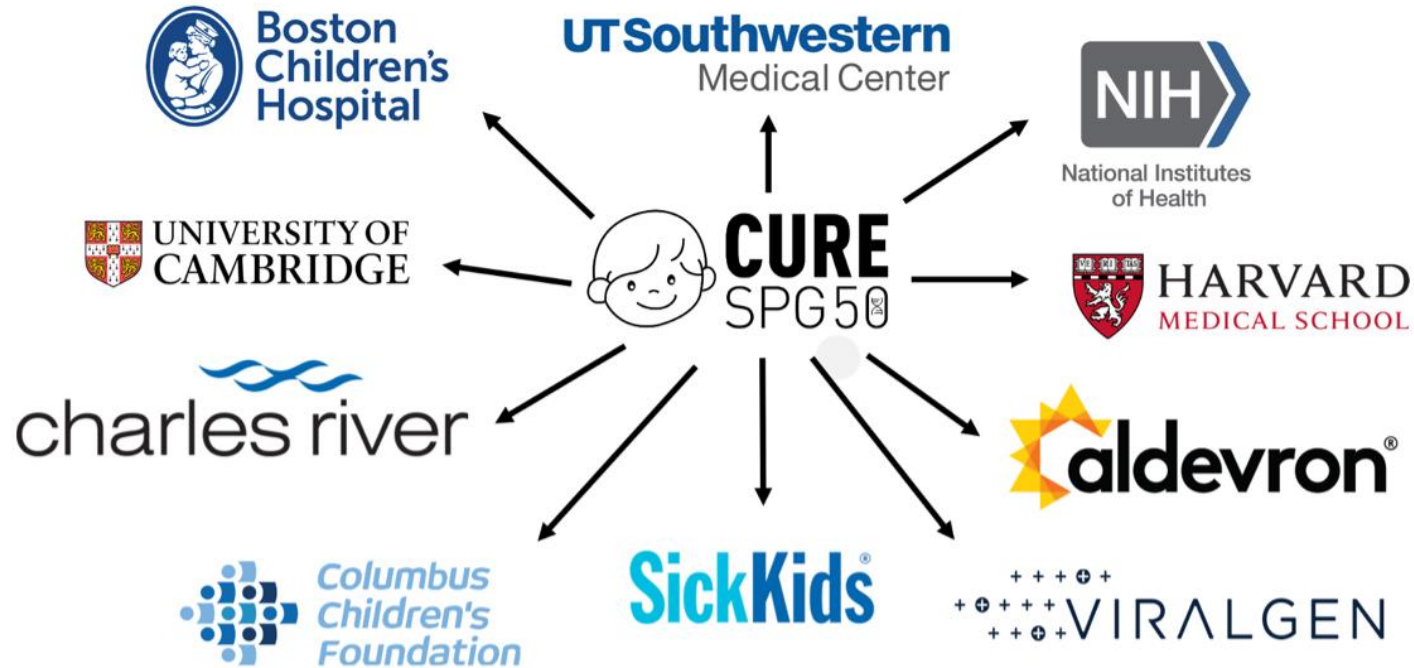


Dr. Xin Chen

\$4.5M Raised
3 years



Patient Advocacy



The Company Organization

Elpida Therapeutics is a Social Purpose Corporation registered in California

The Consortium Approach

- Industry/Philanthropy/Academia/FDA/NIH
- Phase 1-Pivotal
- Disease Programs using AAV viral vector technology
- Clinical development to BLA approval
- Pediatric monogenetic diseases with no cures

Our Pipeline

May 16, 2023

The Foundation for the National Institutes of Health Announces Selection of Eight Rare Diseases for the Bespoke Gene Therapy Consortium Clinical Trial Portfolio

FNIH News Release

June 19, 2023

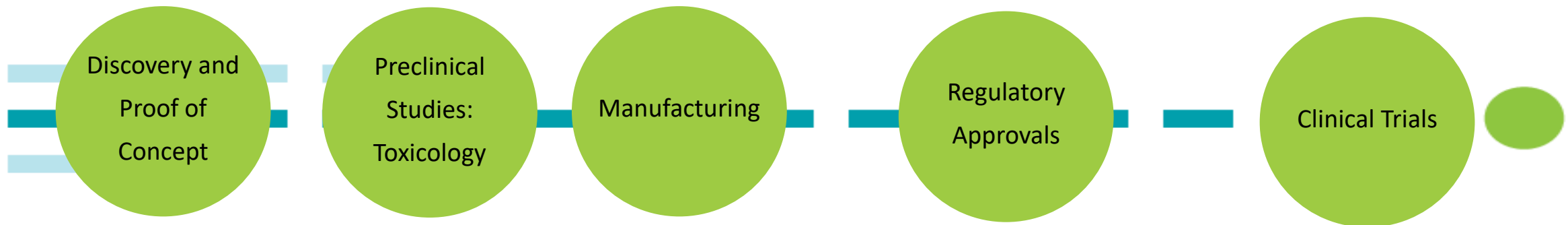
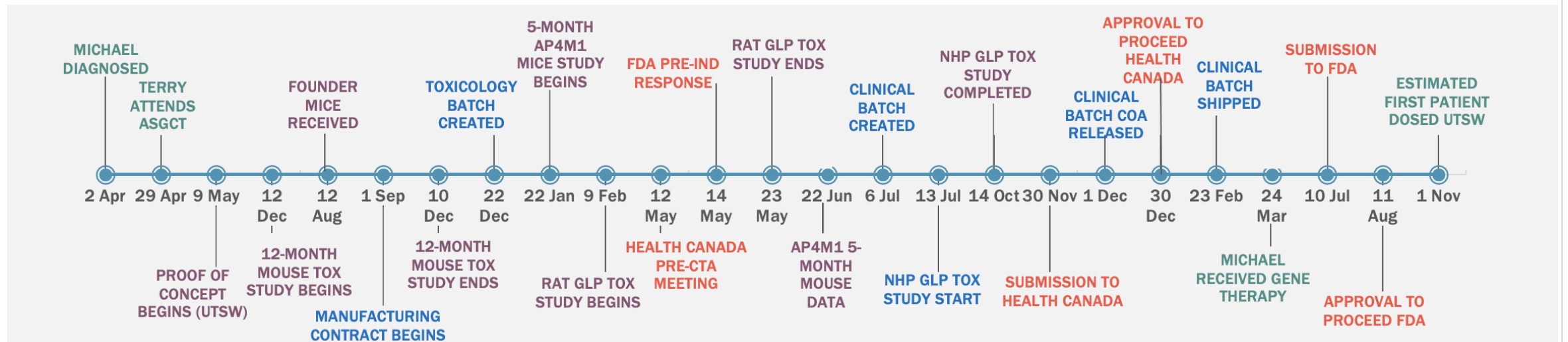
Public-Private Alliance Chooses Eight Rare Diseases for Focused Gene Therapy Development

BioSpace

Pipeline

Program	Indications	Discovery	Pre-Clinical	Phase ½	Pivotal
Melpida	SPG50	Progressing	Progressing	Progressing	Not Started
Elpida-02	CMT4J	Progressing	Progressing	Not Started	Not Started
Elpida-03	TBD	Progressing	Progressing	Not Started	Not Started
Elpida-04	TBD	Progressing	Progressing	Not Started	Not Started
Elpida-05	TBD	Progressing	Progressing	Not Started	Not Started

Pathway to Gene Therapy





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Thank You

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