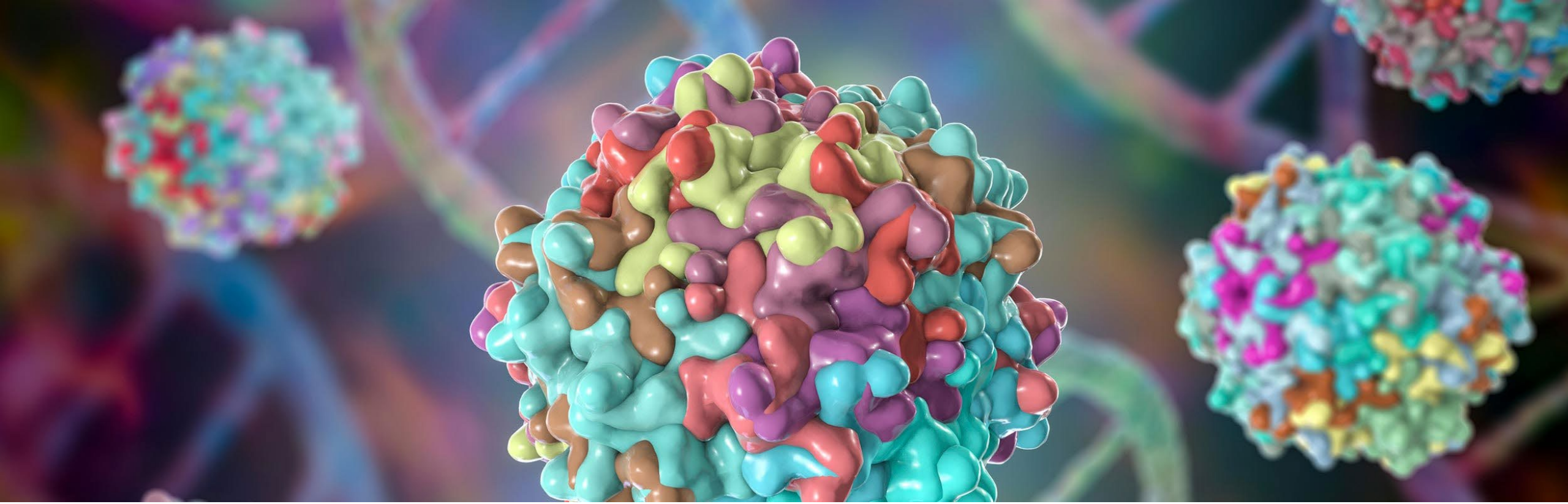


Gene Therapy for CLN7 Disease
CLN7 Community meeting

Jan 18th, 2024

ELPIDA THERAPEUTICS

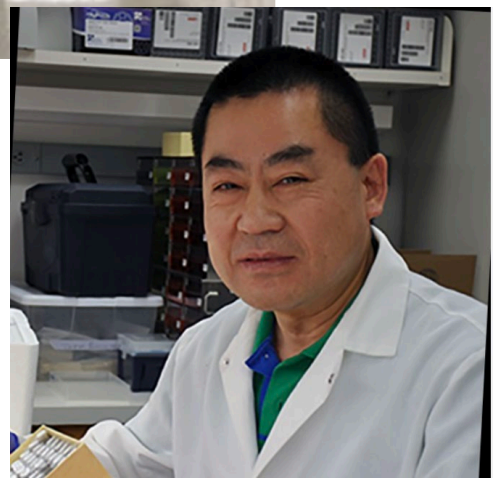
Introductions

Elpida Therapeutics, CEO and Founder
Mr Terry Pirovolakis,

Elpida Therapeutics Team
Dr Souad Messahel
Dr Keith Gottlieb
Rachel Thomas

Elpida Therapeutics Consultants
Dr Diane Balderson
Dr Trey Putman
Dr Susan Walker

Bench to Bedside

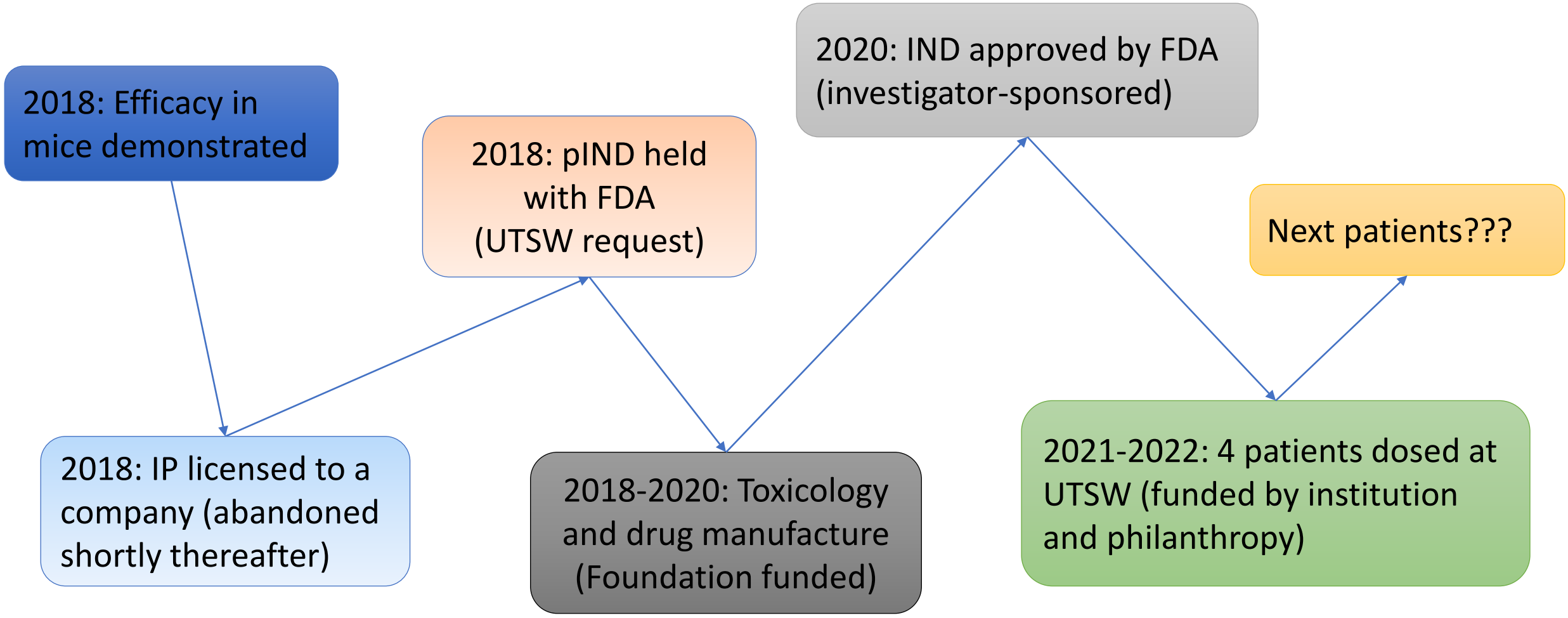


- AAV9 Viral Vector technology led by Dr Steven Gray
- Clinical Development led by Dr Berge Minassian
- Preclinical work completed by Dr Xin Chen
- Pediatric Neurogenetic diseases with no cures
- Highest doses of Gene Therapy given to humans to date

CLN7 Batten's Disease

- Among the rarest form of Battens Diseases
- Dementia, vision loss, epilepsy and early death
- Jojo's story
- Batten's Hope – Gina Hann
- AAV9/MFSD8
- Phase 1- First in Human Clinical trial
- Funded by Children's Health and UTSW, supported by Batten Hope and Mila's Miracle
- No federal or Industry funding

Translational path for CLN7



Study Design

- Phase 1 first-in-human, single center, open-label, dose escalation study of AAV9/MFSD8
- UT Southwestern Medical Center / Children's Health/ PI- Dr Benjamin Greenberg
- Single-administration study with a planned cohort size of four (4) participants
- Intrathecal dose of AAV9/MFSD8
- 2 Dose levels 5xE14 vg (1 patient) and 1xE15 vg (3 patients)
- ClinicalTrials.gov Identifier: NCT04737460

Primary Objectives

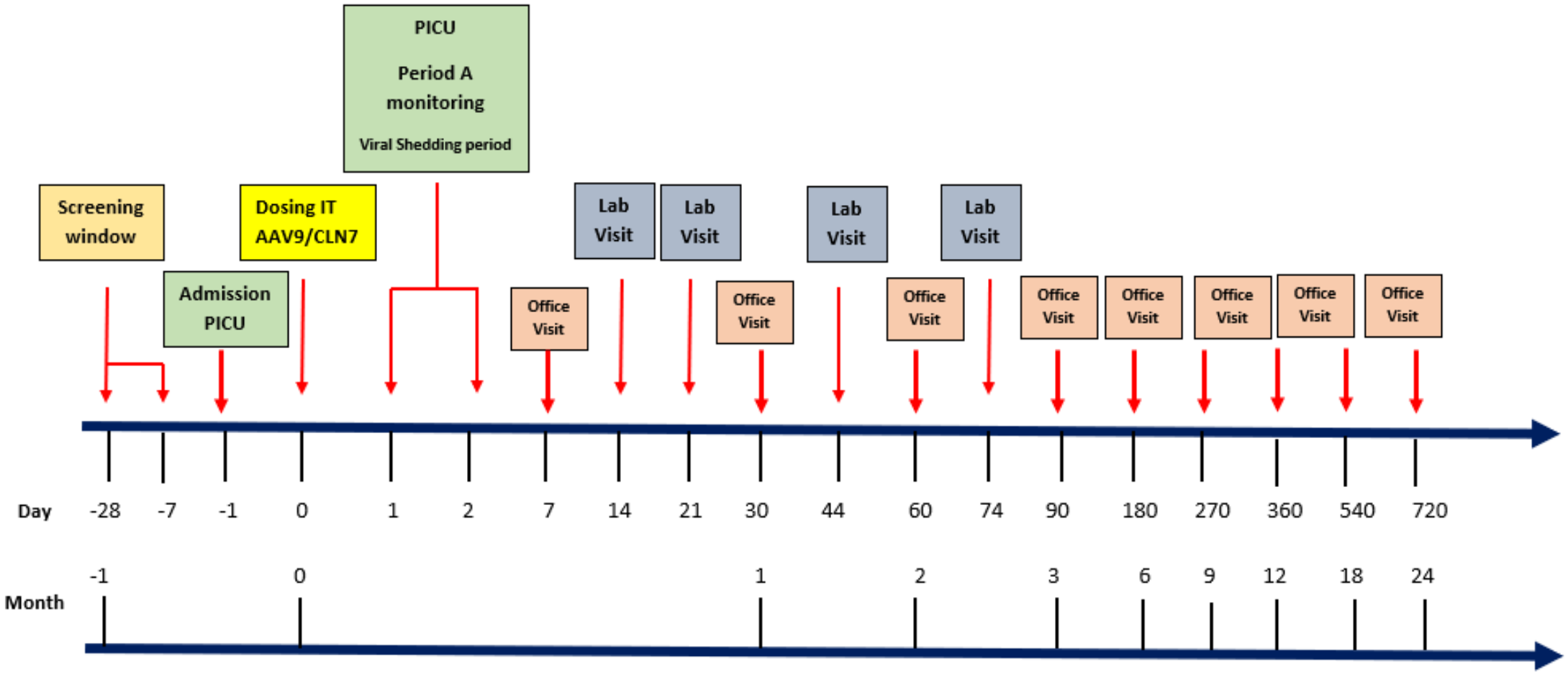
- Safety and tolerability of single doses of AAV9/CLN7 administered intrathecally to children with CLN7 disease
- By the incidence and severity of treatment related serious adverse events (SAEs).

- Period A- 24-month
  - Complete blood profiles
  - MRI brain scans
  - Cerebral spinal fluid analysis
  - Nerve conduction studies
  - Neurologic exam
- Period B- 3-year FU period
  - Annual visits
  - Sirolimus wean
  - 30-month MRI/LP

Secondary Objectives

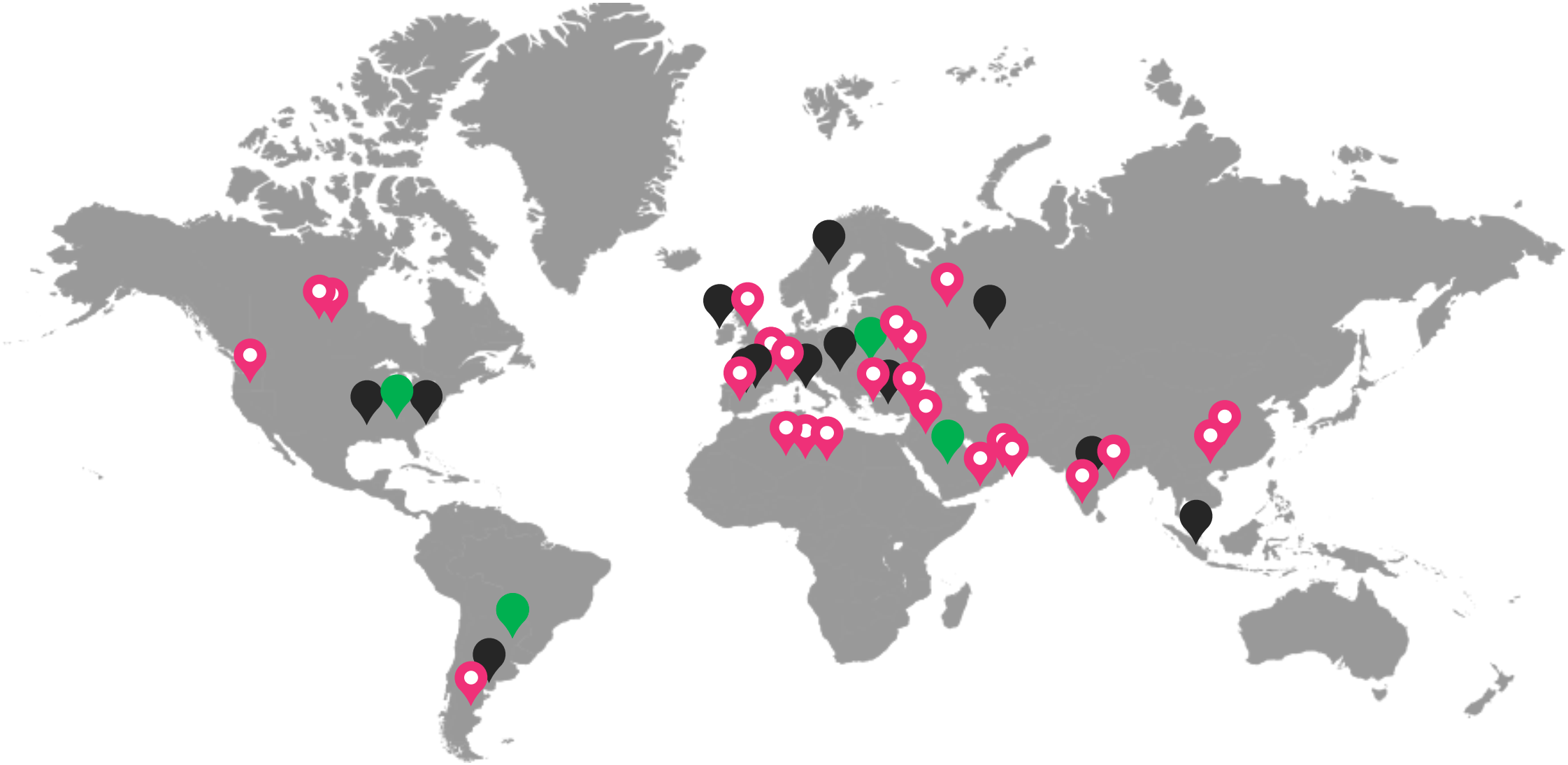
Purpose	Assessment
Disease Burden Scale	1. Clinical Global Impression
Motor Function Assessments	1. 2-Minute Walk Test Or 6-Minute Walk Test 2. Pediatric Balance Scale 3. Gross Motor Function Measure (GMFM)
Intelligence and Cognition Assessments	1. Mullen Scale of Early Learning 2. Vineland Adaptive Behavior Scales, 3rd Edition
Quality of Life Assessments	1. Quality of Life Inventory-Disability (QI-Disability) 2. Infant/Toddler Quality of Life Questionnaire (ITQOL)
Surrogate Measures	1. EEG 2. MRI Brain

Patient visit schedule



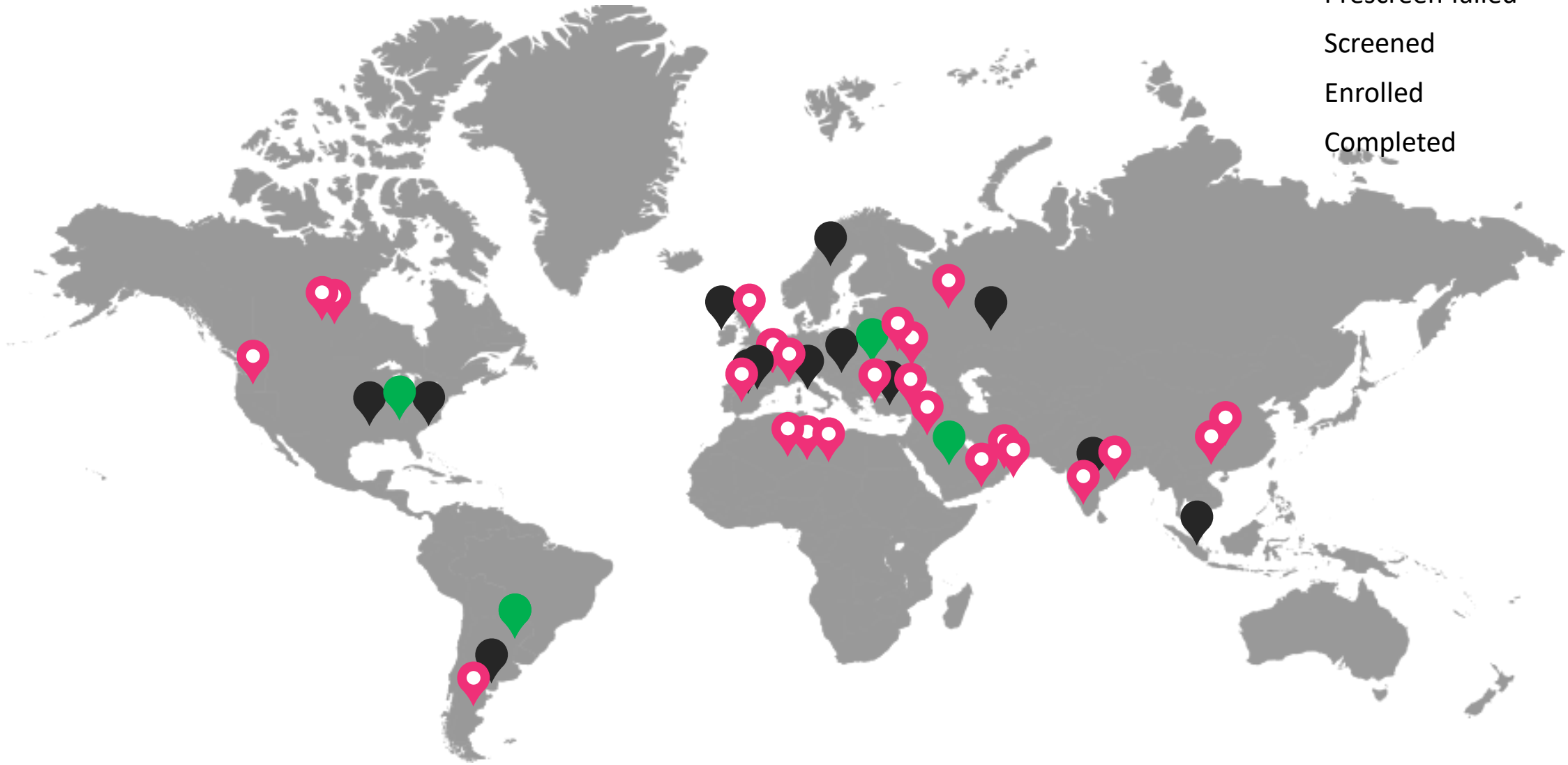
CLN7 referrals

Eligible candidates

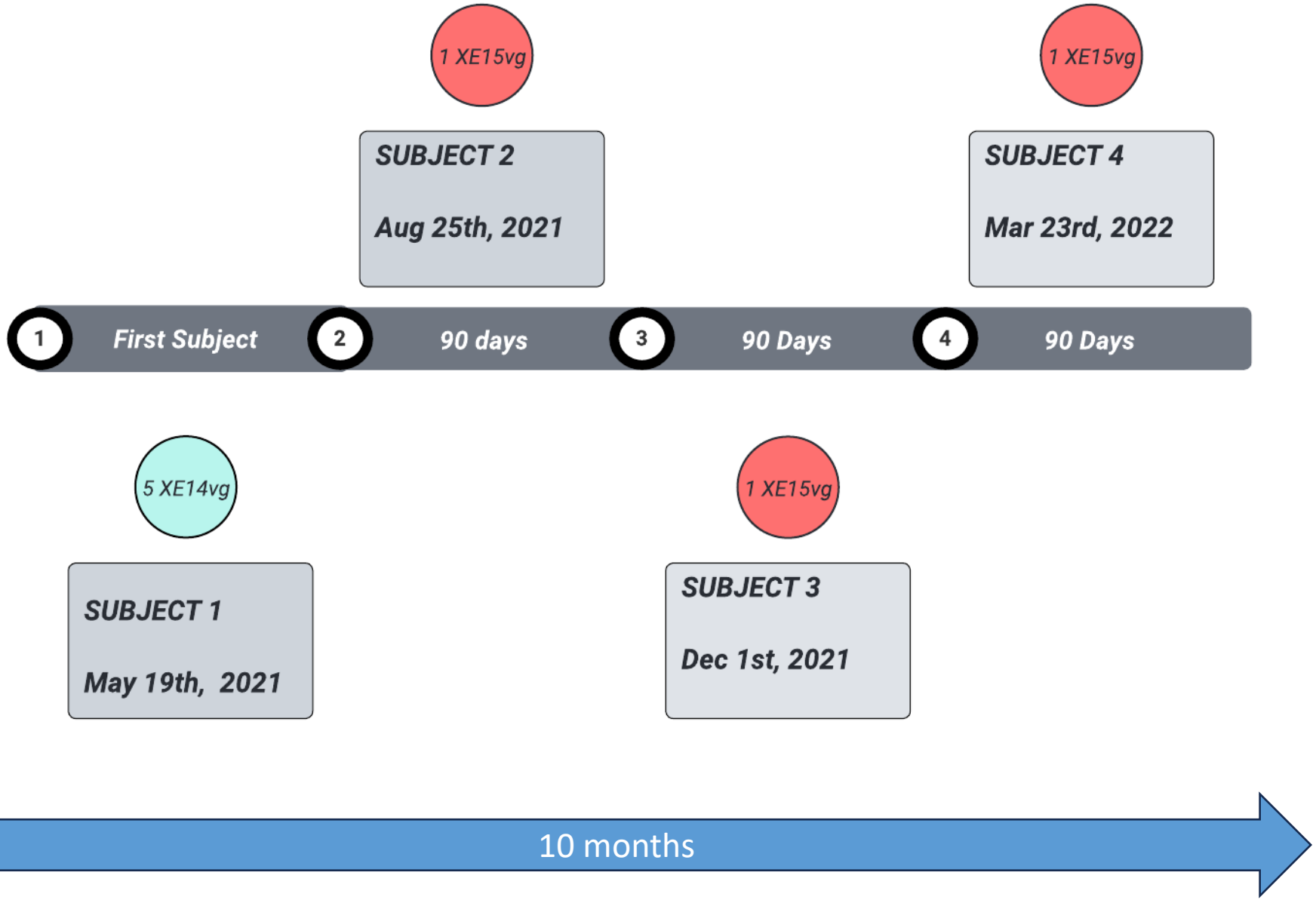


Eligible candidates

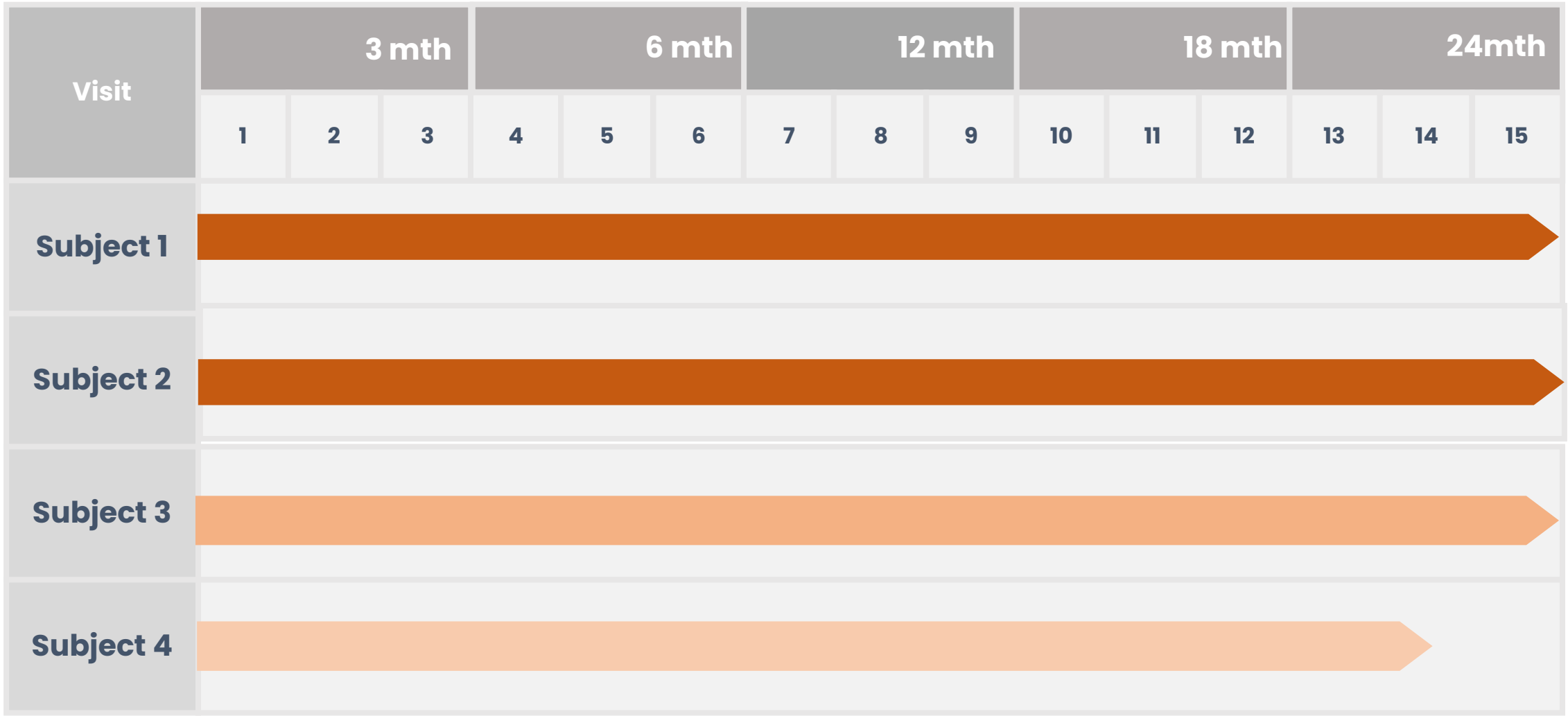
Group	Number
Target number	4
Prescreened	21
Prescreen failed	12
Screened	4
Enrolled	4
Completed	2



Subjects enrolled



Current status of clinical trial



Important results

- No clinically deleterious immune responses
- No evidence of DRG toxicity.
- Nerve conduction has been stable or improved following gene transfer
- No signs of liver injury
- No signs of bone marrow suppression
- MRI brain did not reveal any signs of inflammatory response

IT clinical trials to date

Disease	Company	ClinicalTrials.gov	Status	Vector	Phase	Dose	Patients Treated	Age Range
Spastic ParaplegiaType 50 SPG50	Elpida Therapeutics	NCT06069687 (The Hospital for Sick Children) NCT05518188 (UTSW Medical Center)	NCT06069687 (Active, Not Recruiting) NCT05518188 (Recruiting)	MELPIDA (scAAV9-hAp4m1opt)	Phase I/II	1E15 Total	4	4 Months-10 Years
CLN7 Batten Disease CLN7	UTSouthwestern Medical Center	NCT04737460 (UTSW Medical Center)	Active, Not Recruiting	AAV9/CLN7	Phase I/II	1E15 Total	4	4-6years
Rett Syndrome	Taysha	NCT05606614 (Taysha)	Recruiting	TSHA-102 (scAAV9-miniMECP2)	Phase I/II	Cohort 1: 5E14 Cohort 2: 1E15	2	>18 Years
Giant Axonal Neuropathy GAN	Taysha/NIH	NCT02362438 (Taysha)	Recruiting	scAAV9-JeT-GAN	Phase I/II	Cohort 1: 1.2E14 Total Cohort 2: 1.8E14 Total Cohort 3: 3.5E14 Total	14	>3 Years
GM2 Gangliosidosis GM2	Taysha	NCT04798235 (Queen's University)	Active, Not Recruiting	TSHA-101 (AAV9/HEXA and HEXB, bicistronic vector)	Phase I/II	5E14 Total	2	Up to 15 Months
Spinal Muscular Atrophy (with Respiratory Distress) SMARD1	Alcyone Therapeutics	NCT05152823 (Nationwide Children's Hospital)	Enrolling by Invitation	ACTX-401 (AAV9/IGHMBP2)	Phase I/II	1E13 Total	6	2 Months - 14 Years
Spinal Muscular Atrophy SMA	Novartis Gene Therapies	NCT03381729 (Novartis Gene Therapies)	Terminated	AVXS-101 (scAAV9/SMN) [Zolgensma]	Phase I/II	Cohort 1: 6E13 Total Cohort 2: 1.2E14 Total Cohort 3: 2.4E14 Total	25	6 Months -2 Years 2 Years -5 Years
CLN3 Battens CLN3	Amicus	NCT03770572 (Amicus Therapeutics)	Active, Not Recruiting	AT-GTX-502 (scAAV9/CLN3)	Phase I/II	6E13 Total	7	3 Years – 10 Years
CLN6 Battens CLN6	Amicus	NCT02725580 (Amicus Therapeutics)	Completed with results	AT-GTX-501 (scAAV9/CLN6)	Phase I/II	1.5E13 Total	13	>1 Year

ELPIDA THERAPEUTICS

Gene Therapy Programs for Ultra-rare Diseases to advance with ground-breaking launch of Elpida Therapeutics SPC

NEWS PROVIDED BY
Elpida Therapeutics, SPC
May 09, 2023, 16:08 GMT

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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

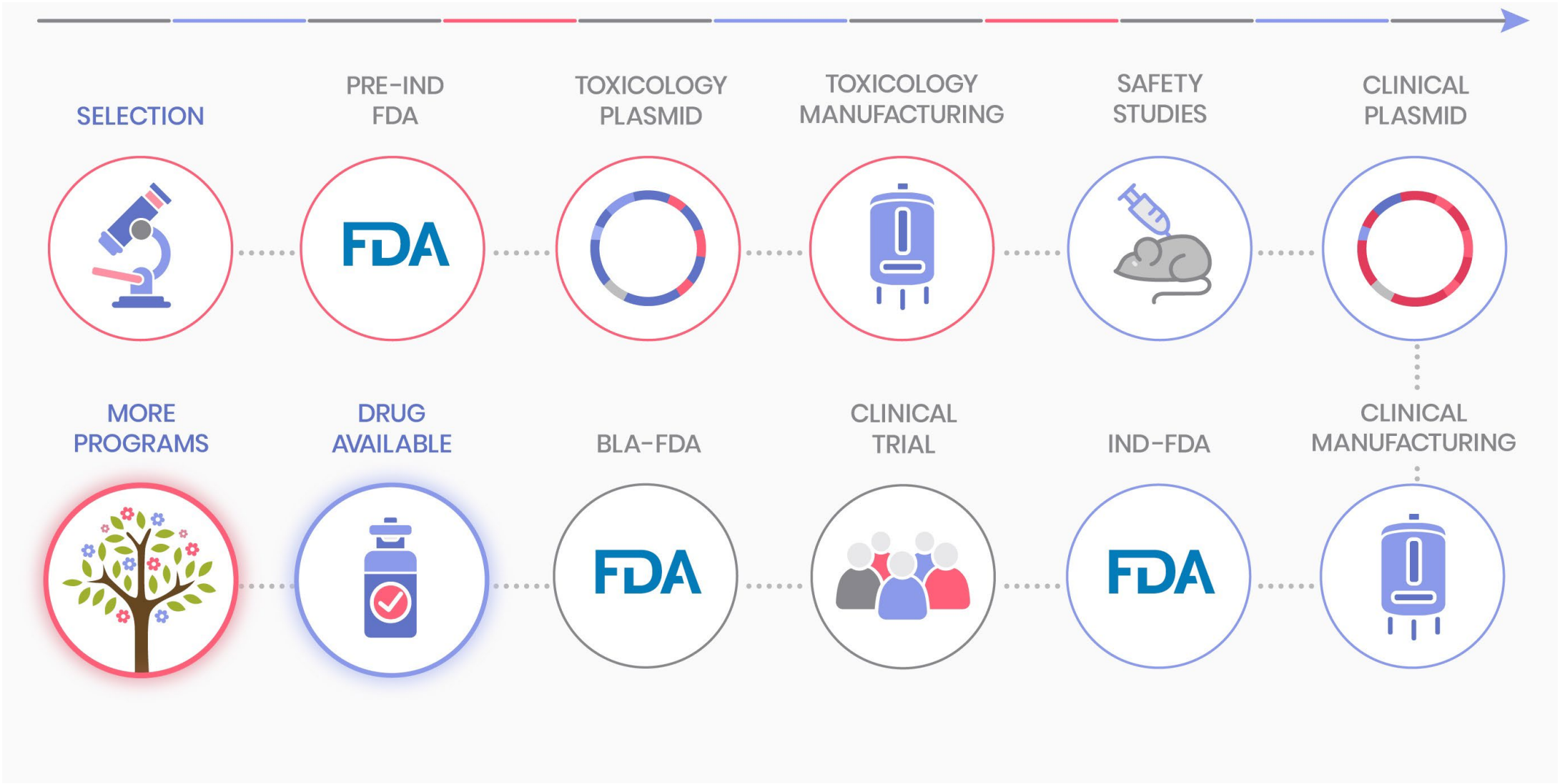
The Company Organization

Elpida Tx 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

Process Flow

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charles river
NIH
Cedars Sinai
UT Southwestern Medical Center
Center for Breakthrough Medicines
CHOC
Spark THERAPEUTICS
ultragenyx pharmaceutical

Next steps- discussion

- Active FDA IND
- Pivotal stage – BLA enabling trials
- 8 to 12 patients
- 2 to 3 US sites

- Elpida Therapeutics – Drug Manufacturing
- Fundraising campaign to pay for hospital costs and travel
- Select newly diagnosed patients
- Natural History data

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