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Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium

COMPLETED SELECTION PROCESS

1 Disease Nomination

- 62 diseases nominated

2 Technical Review

- 3 reviewers perform independent reviews for each application
- Review teams include:
  - Expertise in disease area/organ system involved
  - Expertise in AAV gene therapies
  - Expertise with clinical trials
- Applications are scored against the criteria established in the disease nomination form
- Conflicts of interest avoided

3 Clinical Subteam Review

- Individual technical scores aggregated and discrepancies reconciled
- Strength of criteria indicating clinical trial readiness*
- Recommended applications move to full Steering Committee

4 BGTC Steering Committee Decision

- Subteam recommendations reviewed
- Steering Committee votes on final 12-15 diseases to move to submission of clinical trial applications
- Selected diseases announced and Clinical Trials RFP issued

5 Clinical Trial RFP

- Clinical trial RFP issued, naming 14 eligible diseases and defining the selection criteria

6 Technical Review

- Triplicate subject matter expert reviews
- Preclinical reviews

7 Subteam Review & Portfolio Considerations

- Resolution of discrepancies
- "Portfolio approach" to ensure broad diversity among disease states and potential patient populations participating in clinical trials
- Recommended portfolio to Steering Committee

8 Steering Committee Review & Vote

*Criteria include gene target able to be inserted into an AAV vector; sufficient proof of concept and natural history studies; established disease models—at the gene, organ, or full-animal level—able to demonstrate transgene functionality, dose-finding studies, and conduct safety and efficacy testing; the existence of at least one validated, clinical meaningful outcome measure with suitability for an AAV gene therapy clinical trial; a known patient population affected by the disease and supported by a patient advocacy group; and not commercially viable.

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FINAL CLINICAL TRIAL PORTFOLIO

Disease type	Disease Name (pseudonym)	Affected Gene	Lead Institution Conducting Clinical Trial
Ocular	Congenital Hereditary Endothelial Dystrophy (CHED)	SLC4A11	University of California Los Angeles
	Retinal Degeneration (NPHP5)	NPHP5	University of Pennsylvania
	Retinitis pigmentosa 45 (RP45) - CNGB1	CNGB1	Columbia University
Neurological	Multiple Sulfatase Deficiency (MSD)	SUMF1	Children's Hospital of Philadelphia
	Charcot-Marie-Tooth disease type 4J (CMT4J)	FIG4	Elpida Therapeutics & University of Texas Southwestern
	Spastic paraplegia 50 (SPG50)	AP4M1	Elpida Therapeutics & University of Texas Southwestern
Systemic	Propionic Acidemia (PA)	PCCB	National Human Genome Research Institute
	Mucopolysaccharidosis IVA (MPS IVA, Morquio A Syndrome)	GALNS	Nemours Children's Health

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