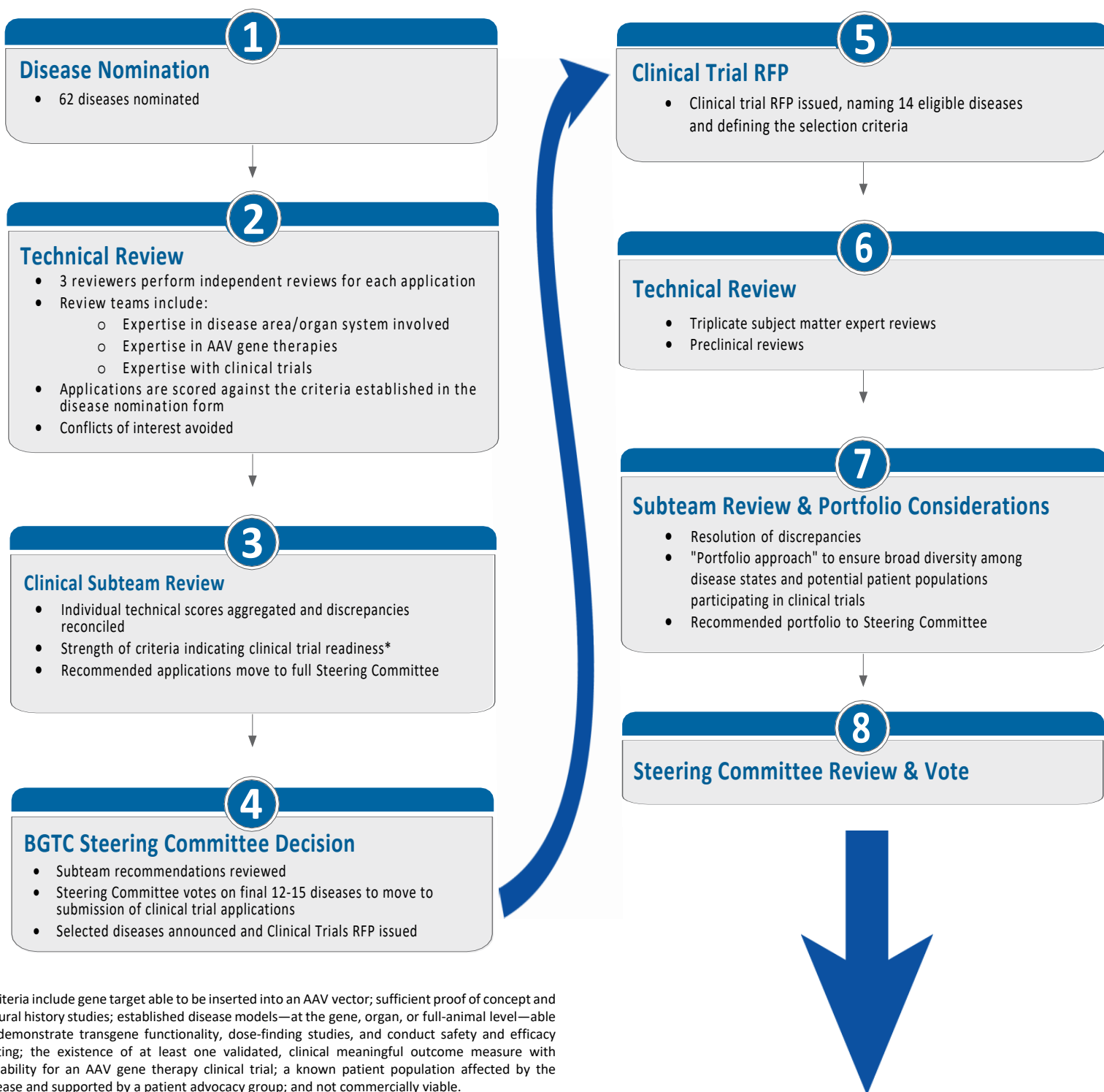






## COMPLETED SELECTION PROCESS





## FINAL CLINICAL TRIAL PORTFOLIO

| Disease type        | Disease Name (pseudonym)                                | Affected Gene | Lead Institution Conducting Clinical Trial             |
|---------------------|---|---------------|--|
| <b>Ocular</b>       | 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                                    |
| <b>Neurological</b> | 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 |
| <b>Systemic</b>     | 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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