ATSENA THERAPEUTICS

Corporate Presentation

September 2021

Bringing life-changing power of genetic medicine to reverse or prevent blindness

Gene therapy has potential to treat the most common forms of inherited blindness

- Patient focused
- Clinical-stage company
- Strong pipeline of preclinical programs
- Focus on inherited retinal diseases
- Novel AAV technologies and capsids
- Experienced team with strong backing

Experienced Founders & Management Team

Management Team

- Patrick Ritschel, MBA- CEO
  - Stride Bio, Bamboo Therapeutics, Dupont, Monsanto
- Kenji Fujita, M.D. - CMO
  - Alnylam, Alexion Pharmaceuticals, Merck, McKinsey & Company
- Linda Couto, Ph.D. - CSO
  - Spark Therapeutics, Children's Hospital of Philadelphia, Avigen, Benitec, Somatix
- Mike Kelly, M.Sc.- Vice President CMC
  - Avrobio, Biogen, Sanofi Genzyme, Cell Genesys
  - Chair Standards Co-Ordinating Body, Gene Therapy

Experienced Founders

- Shannon E. Boye, Ph.D.- Founder and Director
  - Professor of Pediatrics
  - Associate Chief, Division of Cellular and Molecular Therapy
  - University of Florida
- Sanford L. Boye, M.Sc.- Founder and CTO
  - Associate Scientist, Director of Vector Core
  - Powell Gene Therapy Center, Department of Pediatrics
  - University of Florida

Clinical Advisory Board

Dr. Cristine Kay

Dr. Jose Sahel

Dr. Mark Pennesi

Dr. Andreas Lauer

Dr. Kim Stepien

Well-positioned for success

- Raised over $60 million in 2020
- Investors with strong track records in biotechnology and gene therapy
  - Foundation Fighting Blindness, Hatteras Venture Partners, Sofinnova Investments, Abingworth, Lightstone Ventures, Osage University Partners, University of Florida and the Manning Family Foundation
- Growing team of over 30 people as of today
  - Continuing to hire across the organization
  - Bringing in specific expertise in ocular gene therapy, clinical and regulatory development
- Established corporate labs & offices
  - R&D, manufacturing facility being outfitted in North Carolina
  - Clinical team established in NY/NJ

A Leading Ophthalmic Gene Therapy Company: Strong Clinical and Preclinical Assets & Platform

Atsena created around game-changing assets, unique discovery platform and robust IP from seminal gene therapy work at the University of Florida

Clinical Stage Program: Leber congenital amaurosis 1 (LCA1)

- Acquired from Sanofi July 2020
- Ongoing Phase 1/2 clinical trial evaluating gene therapy
- Backed by 15+ years research, preclinical evidence from founders' labs at University of Florida

Rapidly Advancing Preclinical Programs

- *MYO7A* Usher syndrome (USH1B) dual-AAV vector treatment to prevent blindness
  - Backed by 8+ years research, preclinical work from founders' labs University of Florida
- **Undisclosed second preclinical program** for inherited retinal disease
- Both programs are 1 -2 years from clinic

Differentiated Technology Platform

- Novel AAV vectors engineered to overcome the unique hurdles presented by retinal disease

Atsena's USH1B Development Program – Status & plans forward

- In-licensed technology from the University of Florida in 2020
- Currently working on lead candidate selection
  - Selecting the best capsid and transgene combination
- Following lead candidate selection,
  - Dose-ranging studies
  - GLP-Tox study
  - Assays & Reagents - preclinical development work ongoing
    - Establishment of critical assays
    - Generation of critical reagents
  - CMC preparations – making clinical grade vector
    - Increased emphasis on CMC attributes by FDA/EMA (quality, testing of vector)
    - Complexities of dual vector approach
- IND Filing
- First in Human clinical trial

Thank you