Director, Translational Research

Atsena Therapeutics is a clinical-stage gene therapy company that is leveraging novel AAV vectors designed to overcome the unique hurdles presented by retinal disease to reverse or prevent blindness. Our lead program, ATSN-201, is a gene therapy for X-linked retinoschisis (XLRS), which is currently being evaluated in a Phase 1/2 clinical trial. We are also advancing ATSN-101, a gene therapy for the treatment of *GUCY2D* Leber congenital amaurosis (LCA1) towards a Phase 3 trial. We have additional preclinical programs to treat other forms of inherited retinal disease as well as novel capsid technology suitable for addressing large indications.

At Atsena, we are bringing patients into focus. We are passionate about finding cures for visually impaired and blind individuals and are driven by cutting edge science to ensure we achieve the safest and most effective results.

Position Summary:

We are seeking a highly motivated and experienced Director, Translational Research, to advance our pipeline. The successful candidate will work with a multi-disciplinary team of scientists to develop therapies that have the potential to restore and preserve vision. The candidate will be responsible for the experimental design, execution and data analysis of all translational research, including non-clinical safety and pharmacology/toxicology profiling. In close collaboration with the CSO, this individual will develop and execute the translational strategy of the company for a variety of pipeline projects and development programs. Working closely with the clinical team and external CROs, this role will also have responsibility for development, qualification, and validation of required clinical assays. The individual will be self-motivated, with a desire to succeed at the highest level. A background in gene therapy is required and a proven ability to deliver novel therapeutic approaches into the clinic is essential.

Responsibilities:

- Lead the translation of discovery research by the design, execution and interpretation of *in vitro* and *in vivo* studies evaluating therapeutic candidates.
- Develop and qualify/validate assays for the analysis of program-related nonclinical and clinical samples.
- Initiate and oversee work performed by external collaborators and CROs.
- Execute, oversee, and interpret IND-enabling efficacy and safety studies.
- Draft, review, edit and finalize non-GLP and GLP nonclinical study protocols, amendments, and study reports, ensuring that documents meet regulatory and compliance requirements, as well as program timelines.
- Author regulatory documents (eg IND filings)
- Present analyses and interpretations of nonclinical studies to regulatory agencies and other oversight bodies in meetings and documents including scientific advice proceedings, IND/CTA applications, annual reports, safety reviews, investigator brochures, and market applications.
- Develop and manage budgets and timelines for key projects, implementing processes and identification of critical program activities/constraints.
- Maintain a current understanding of pharmacology, toxicology and gene therapy literature and methodology, as well as the scientific literature related to the specific indications being supported.

- Present results at scientific conferences and draft manuscripts for publication in peer-reviewed journals.
- Some travel will be required.

Professional Experience and Qualifications:

- Ph.D. or equivalent degree in biomedical sciences, preferably with focus on translational research with 10+ years' experience.
- Substantive experience working with gene therapies. Ocular gene therapy experience preferred.
- Comprehensive experience with biochemistry, cellular biology, and molecular biology and deep experience in translational research.
- Experience in the development of functional potency assays and clinical assays such as ADA
- Demonstrated experience working with animal models and ability to design experiments aimed at predicting clinical outcomes.
- Excellent critical thinking and scientific skills, and a demonstrated ability to analyze, interpret, and clearly communicate complex results.
- Ability to multi-task and manage several projects in parallel, paying attention to detail.
- Extensive knowledge of GLP regulations and reporting requirements.
- Proven ability to work effectively and collaboratively on cross-functional teams, including leading and spearheading projects internally and externally.
- Self-starter with proven ability to thrive in a fast-paced, entrepreneurial yet highly disciplined environment.
- Excellent oral, written presentation and communication skills.

Role Location

- Onsite