

**UNITED STATES DISTRICT COURT
FOR THE DISTRICT OF DELAWARE**

THE TRUSTEES OF THE UNIVERSITY
OF PENNSYLVANIA and REGENXBIO
INC.,

Plaintiff,

v.

SAREPTA THERAPEUTICS, INC. and
SAREPTA THERAPEUTICS THREE, LLC,

Defendant.

C.A. No. 20-1226 (RGA)

JURY TRIAL DEMAND

OPENING EXPERT REPORT OF PAOLA LEONE

Dated: April 28, 2023

By: _____



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At the request of counsel for Plaintiffs REGENXBIO, Inc. and The Trustees of The University of Pennsylvania, I hereby submit the following report pursuant to Federal Rule of Civil Procedure 26(a)(2)(B).

I. QUALIFICATIONS

1. I have attached my *curriculum vitae* as **Exhibit A** to this report. I highlight certain aspects of my educational background, career history, publications, and other relevant qualifications in the summary below.

2. I am the director of the Cell and Gene Therapy Center at Rowan University School of Osteopathic Medicine in Stratford, New Jersey. I have over thirty-five years of research experience studying molecular genetics, including gene therapies, neurogenetics, and relevant pharmacology.

3. I attended the University of Padua, Italy, where I received my Doctorate in Neuroscience in 1987.

4. I have also been a Professor in the Department of Cell Biology and Neuroscience at Rowan University School of Osteopathic Medicine since 2013. Prior to that, I served as the Director of the Cell and Gene Therapy Center at the University of Medicine and Dentistry of New Jersey, School of Osteopathic Medicine. I have also served as a Lecturer and Professor at the Graduate School of Biomedical Sciences at Rowan University as well as at the University of Medicine and Dentistry of New Jersey in the Department of Cell Biology and at the New Jersey Institute of Successful Aging.

5. In addition to my employment in the academic setting, I have served on numerous national and international scientific committees designed to advance gene therapies, including gene therapy for the treatment of Duchenne Muscular Dystrophy.

6. Throughout my career I have received a number of awards and honors, which are listed on my C.V., including the New Jersey Health Foundation Excellence in Research Award, Edward J. Ill Medical Foundation Award in Biomedical Research, and the Innovator Award from the New Jersey Inventor Hall of Fame. I have also been honored numerous times by the International Rare Disease Day, Hall Of Fame for my work.

7. My research over the past twenty years has focused on neuroscience, molecular genetics, and gene therapy applications for the treatment of various human diseases. Some publications relevant to the current subject include: Francis et al., *Molecular Therapy: Methods and Clinical Development* (2021); Janson et al., *Neurosurgery* (2014); Leone et al., *Science Translational Medicine* (2012); McPhee et al., *Journal of Gene Medicine* (2006); and Janson et al., *Human Gene Therapy* (2002). These papers, and many others listed in my C.V., are the result of my long-standing research investigations concerning molecular genetics and the use of gene therapies for the treatment of human diseases.

8. Among my other accomplishments in the field of gene therapy, in 2001, I served as Investigational New Drug (IND) Sponsor as well as Research PI in an NIH-funded Phase I/II clinical study designed to develop a “Gene Therapy Treatment for Canavan Disease” using AAV2-ASPA in collaboration with Children’s Hospital of Philadelphia (PA), Cooper University Hospital (NJ), NYU Hospital (NY), and the University of North Carolina (NC). This project entailed the first clinical application of Adeno-Associated Viral Vectors in the human brain for a neurologic disorder, as part of an international Phase I/II clinical trial with a minimum of five years follow-up on each patient and provided a foundation for brain gene therapy for future clinical applications. The therapy was well tolerated and provided benefit to patients as reported in Leone et al., *Science Translational Medicine*. (2012).

9. My recent work focused on the development of a novel next-generation gene therapy technology directed to target and repair white matter and prevent disease progression in Canavan Disease that could be applicable to the treatment of other leukodystrophies. The gene therapy targeting white matter using AAVolig001-ASPA is currently in Phase I/II (ClinicalTrials.gov Identifier: NCT04833907).

II. FEES

10. I have been retained by Fish & Richardson P.C. as an expert witness for Plaintiffs REGENXBIO, Inc. and The Trustees of The University of Pennsylvania (“Plaintiffs”) in this litigation. I am being compensated for the services I render in this case at an hourly rate of \$700 plus expenses. My compensation is in no way based on the outcome of the litigation.

III. PRIOR EXPERT TESTIMONY

11. In the last four years, I have not testified as an expert at trial or by deposition.

IV. MATERIALS CONSIDERED FOR THIS REPORT

12. I have been asked to provide my opinions relating to infringement of U.S. Patent No. 10,526,617 (“the ’617 patent”) by Sarepta.

13. In formulating my opinions and writing this report, I have reviewed portions of the materials listed in Exhibit B. I reserve the right to rely on additional materials produced in this litigation after the submission of my report and to respond to any expert reports submitted by Sarepta.

14. My opinions expressed in this report are based on those materials as well as my general knowledge, education, and experience.

V. SUMMARY OF OPINIONS AND EXPECTED TESTIMONY

15. I understand that Plaintiffs have asserted infringement of claims 1-9, 12, 15, and 18-25 of the ’617 patent. I will refer to those claims as the Asserted Claims.