



APIE Therapeutics, Inc

2 Davis Dr Research Triangle Park
RTP, NC 27709
www.apie-therapeutics.com

Leaders in Harnessing the Apelin-APJ Signaling
Pathway for Successful Treatments of Chronic



Industry: Biotech/Pharma

- Initial Target Indication: Scleroderma-Interstitial Lung Disease SSc/SSc-ILD

Management

- Esther M. Alegria, Ph.D.
Chief Executive Officer
- Robert Willette, Ph.D.
Chief Scientific Officer
- Rajesh Manchanda, Ph.D.
Chief Technical Advisor
- Seth Hetherington, MD
Chief Medical Officer
- Debra Bowes, MBEE
Chief Business Officer

Board of Directors

- Maureen O'Connor, JD, Chair
- Peter Johnson, MD
- Diane Jorkasky, MD
- Seth Hetherington, MD
- Esther Alegria, Ph.D.

Scientific Advisory Board

- John Varga, MD
Rheumatology / SSc, Univ of Michigan Med School
- Shelia Violette, Ph.D.
Immunology/inflammation
Chief Scientific Officer, Q32Bio
- Sudar Rajogopal, MD, Ph.D.
Cardiovascular/GCPRs
Duke Medical Center
- Jonathan Krosky, MD
Pulmonary/Pathophysiology
Univ of Vanderbilt Medical Center
- Neeraj Dhaun, MD, Ph.D.
Kidney Clinical Research
Univ of Edinburgh-UK

Funding to Date

- \$7M Non-Diluted (Pre-Seed)
- \$1.4M Diluted (Bridge)

Intellectual Property

- Exclusive worldwide license to all technologies
- 3 patent portfolios issued covering composition of matter, methods of treatment through 2042
- APT101 (exp 2037); APT102 (2042)
- Multiple countries covered

Seeking a \$6M (Seed)

- File IND SSc/SSc-ILD (12 months)
- Secure Clinical Site

Next funding round \$25M (Series A)

- Ph1a/1b, POC
- Enabling Ph2/3 Pivotal Trials

Executive Summary:

- APIE Therapeutics (APIE-Tx) is a pre-clinical-stage small molecule platform company focused on diseases impacted by microvasculature endothelium breakdown in different organs.
- Advances in chronic, immune-mediated fibrotic diseases pathophysiology have established microvascular injury/dysfunction as precursor of disease progression.
- Harnessing the APJ Receptor signaling pathway leads to repair and regeneration of the damaged microvasculature, and inhibits fibrotic progression, which can potentially improve the health and long-term outcome in patients.
- The APJ Receptor is expressed in the endothelium microvasculature cell types in the lungs, kidneys, skin, brain, and vasculature and is overexpressed upon disease progression in such specific cell types.
- APIE-Tx has exclusively licensed potent and highly selective Apelin/APJ agonists. Rigorous medicinal chemistry efforts developed a small molecule library of over 800 Apelin/APJ agonist compounds with unique properties for a variety of disease targets.

Market Opportunity/Unmet Need:

Systemic Sclerosis / SSc-Interstitial Lung Disease (SSc-ILD)

- SSc is a progressive systemic disease of unknown cause characterized by **fibrotic scarring in major organ systems** (skin, lungs, kidneys).
- SSc-ILD (major cause of early death **approx. 5 years life expectancy** from diagnosis and represents a large unmet medical need).
- Two approved drugs have **limited efficacy and safety issues: Nintedanib (TK inhibitor) and Tocilizumab (IL-6 mAb)**
- US reported prevalence for SSc is approx. 100k patients / SSc-ILD is approx. 25k patients.
- There is currently no cure for SSc and patients are in urgent need of new drugs to significantly **improve health outcomes; reduce side-effects; extend lifespan**

APIE Therapeutics Pipeline:

Initial Indications

Drug	Primary Indication	Research	Pre-Clinical	IND	Ph 1	Ph 1b/2a
APT-101	Systemic Sclerosis (SSc/SSc-ILD)	→				
APT-101	Idiopathic Pulmonary Fibrosis (IPF)	→				
APT-102	Kidney Nephrotic Syndrome	→				

Technical Milestones Achieved:

- Preclinical evaluation of APT101, APT102 and APT103 Apelin/APJ agonists has confirmed favorable drug-like pharmacological profiles and efficacy in various chronic and fibrotic disease *in-vivo* models.

APT101 Clinical Candidate:

- Extensive PK/metabolic profiling data; no toxicity observed in preclinical model; no-off target safety profile confirmed; preclinical proof of biology and efficacy demonstrated *in-vivo* disease models for acute and chronic lung fibrosis and *ex-vivo* human co-culture cells disease models (e.g. dermal fibroblast/bronchial epithelial cells and renal proximal tubule epithelial Cells/ fibroblasts cells).
- Orphan Drug Application and Pre-IND Meeting on Target 1H2022.
- Manufacturing scale-up has been successfully completed; GMP manufacturing on-going.