



## APIE Therapeutics, Inc

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



### Industry: Biotech/Pharma

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

### Management

- Esther M. Alegria, Ph.D.  
Chief Executive Officer
- Debra Bowes, MBEE  
Chief Business Officer
- Seth Hetherington, MD  
Chief Medical Officer
- Michelle Higgin, Ph.D.  
Chief Regulatory & Dev Officer
- Robert Willette, Ph.D.  
Chief Scientific Officer

### Board of Directors

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

### Scientific Advisory Board

- Neeraj Dhaun, MD, Ph.D.  
Univ of Edinburgh-UK
- Jonathan Krosky, MD  
Univ of Vanderbilt Medical Center
- Rajesh Manchanda, Ph.D.  
Chief Dev Officer, Anokion
- Sudar Rajogopal, MD, Ph.D.  
Duke Medical Center
- John Varga, MD  
Univ of Michigan Med School
- Shelia Violette, Ph.D.  
Chief Scientific Officer, Q32Bio

### Funding to Date

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

### Intellectual Property

- Exclusive WW license to all technologies
- 3 patent portfolios issued covering composition of matter, methods of treatment through 2042
- APT101 (exp 2037); APT102 (2042)
- All Major plus Tier2 Markets covered

### Seeking a \$6M (Seed)

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

### Next funding round \$25M (Series A)

- 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 the understanding of chronic, immune-mediated fibrotic disease 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 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

### Market Opportunity/Unmet Need:

#### Systemic Scleroderma (SSc) / 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 / \$3.8M by 2026
- 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 Scleroderma (SSc)-SSc-Interstitial Lung Disease	→				
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.