

We are a clinical-stage, leading mRNA therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction. Using our proprietary mRNA therapeutic platform (MRT), we create mRNA carrying instructions to produce proteins for therapeutic benefit.

<b>1<sup>st</sup></b> to clinic pan-mutational mRNA therapeutic for <b>cystic fibrosis</b>	<b>Pulmonary Focus</b>  Expanding on our robust lung delivery platform	<b>Powerful Platform</b>  to fuel additional targets	<b>10+</b> Years of mRNA R&D 
<b>Industry-leading Manufacturing</b>  Unique, highly scalable process supported by strategic agreement with global CMO	<b>Broad Patent Portfolio</b>  130+ issued 450+ pending	 Partnership with world leader in vaccine R&D <b>Sanofi Pasteur</b>	<b>Leadership who can execute,</b> involved in development of <b>6</b> approved products

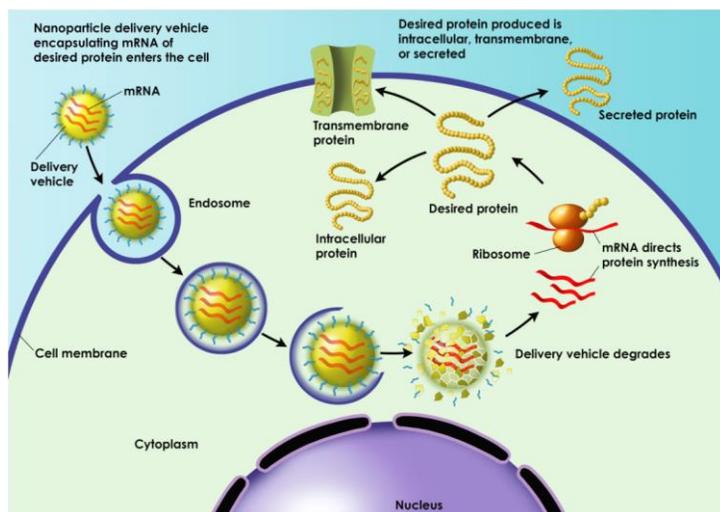
*“Our goal is to deliver innovative medicines to patients using our proprietary MRT platform, that has been under development for more than 10 years and has potential applicability to a broad range of diseases.”*

— Ron Renaud  
Chief Executive Officer

## Our Goal: Develop mRNA Medicines to Restore Protein Function

Using our proprietary MRT platform, we create mRNA that encodes fully functional proteins. When our mRNA is delivered to the target cell, the cell's own machinery recognizes and translates it, restoring or augmenting protein function to treat or prevent disease.

We are primarily focused on developing mRNA therapeutics to treat genetic diseases of the lung where there is high unmet need. Additionally, our MRT platform is potentially applicable to a wide range of diseases in which the production of a desirable protein can have a therapeutic effect, including diseases that affect the liver, eye, and central nervous system. MRT may also be applied to produce therapeutic antibodies and vaccines in areas such as infectious disease and oncology.



### Key Leadership: Collectively Participated in Developing 6 Approved Medicines

**Ronald C. Renaud, Jr.**  
Chief Executive Officer  
Idenix, Amgen, Keryx

**Ann Barbier**  
Chief Medical Officer  
Agiros, Shire

**Frank DeRosa**  
Chief Technology Officer  
Shire, Enzon

**Paula Cloghessy**  
Chief HR Officer  
Joule, Interleukin

**Brian Fenton**  
Chief Business Officer  
Shire, Idenix

**Richard Wooster**  
Chief Scientific Officer  
Tarveda, GlaxoSmithKline

**John Schroer**  
Chief Financial Officer  
Allianz, Healthcor

**Michael Heartlein**  
Executive Vice President and Founder  
Shire, TKT

**Paul Burgess**  
Chief Legal Officer  
Civitas, BIND

**Pat Sacco**  
SVP, Technical Operations  
Shire, Biokinetics

### Quick Facts

(01.30.2020)

TICKER

TBIO

EXCHANGE

NASDAQ

IPO DATE

JUNE 2018

EMPLOYEES

90+

HEADQUARTERS

LEXINGTON, MA



## Our Current Areas of Focus

### Cystic Fibrosis

Cystic fibrosis (CF) is the most common fatal inherited disease in the United States, resulting in mucus buildup in the lungs, pancreas and other organs, and mortality is primarily driven by a progressive decline in lung function. There is no cure for CF. According to the NIH, individuals with CF have a lifespan of approximately 30 years. More than 30,000 patients in the United States and more than 70,000 patients worldwide are living with CF. CF is caused by dysfunctional or missing CFTR protein.

**MRT5005 is designed to address the underlying cause of cystic fibrosis (CF), regardless of genetic mutation by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to cells in the lung through nebulization.**

### Additional Pulmonary Diseases

Beyond our CF clinical program, the primary focus of our research efforts is the evaluation of targets in additional pulmonary diseases utilizing our proprietary lung delivery platform. Preclinical research efforts include discovery-stage programs in primary ciliary dyskinesia (PCD), pulmonary arterial hypertension (PAH) and idiopathic pulmonary fibrosis (IPF).

### Other Diseases

We believe that our MRT™ platform may be applied across a broad array of diseases and target tissues via multiple routes of administration. We have an ongoing collaboration with Sanofi Pasteur Inc. to develop infectious disease vaccines using our mRNA technology. Additionally, we are conducting early-stage discovery activities to identify additional potential mRNA therapeutics.

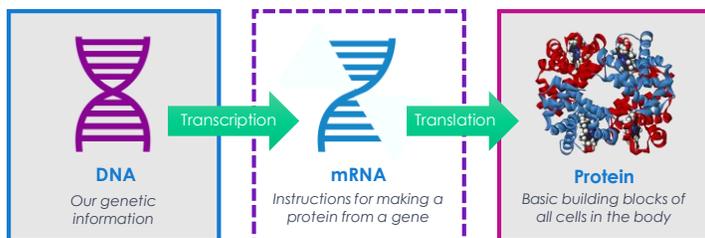
	Focus Area	Indication	Route of Administration	Discovery	IND-Enabling	Phase 1 / 2 Clinical
Direct Therapeutics	LUNG	Cystic Fibrosis (CF)	Inhalation	MRT5005		
		Cystic Fibrosis (CF)	Inhalation			
		Idiopathic Pulmonary Fibrosis (IPF)	Inhalation			
		Primary Ciliary Dyskinesia (PCD)	Inhalation			
		Pulmonary Arterial Hypertension (PAH)	Inhalation			
	LIVER	Undisclosed	Intravenous			
OTHER RARE	Undisclosed	Various				
Vaccines	LYMPHATIC	Infectious Disease	Undisclosed	SANOFI PASTEUR		
		Immuno-Oncology	Various			



In June 2018, we announced a collaboration and exclusive licensing agreement with Sanofi Pasteur to develop mRNA vaccines for up to five infectious disease pathogens which will bring together Sanofi Pasteur's leadership in vaccines and our mRNA research and development expertise.

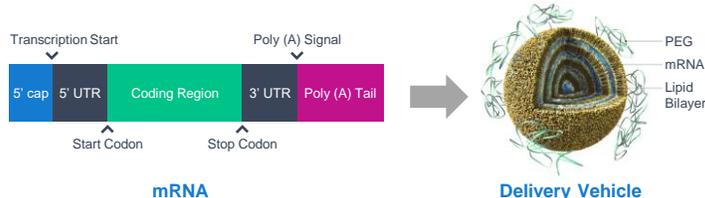
## The Importance of mRNA

mRNA is a fundamental component of gene expression. It is the key link in the process of translating genetic information encoded in DNA into instructions that are used by cells to produce the proteins needed to carry out essential cellular functions. These instructions are processed through cellular mechanisms in two steps: transcription and translation.



By addressing the central step in the production of proteins, our MRT product candidates are designed to give the body the instructions to **make its own** therapeutic protein

## Our MRT Platform



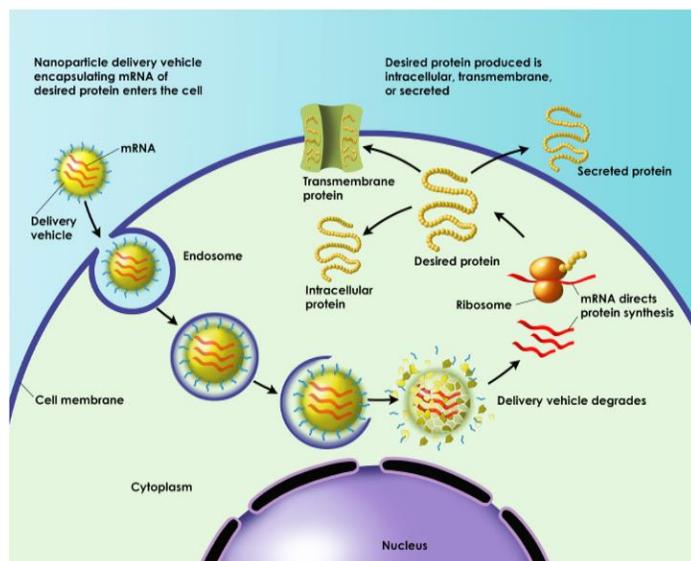
- ✓ Engineered for enhanced stability and optimal expression of desired proteins
- ✓ Proprietary delivery vehicle optimized for specific tissue distribution

Our mRNA therapeutics platform (MRT) consists of two major components: the protein-coding mRNA and a delivery vehicle. Our platform is designed to be flexible and scalable by allowing for the development of product candidates that vary only in the mRNA sequence and the tissue-specific delivery vehicle. This modular nature of our platform may allow us to rapidly advance into new indications.

## MRT has the potential to treat the underlying cause of many diseases by restoring protein expression.

Using our proprietary MRT platform, we create mRNA that encodes functional proteins. When our mRNA is delivered to the target cell, the cell's own machinery recognizes and translates it, restoring or augmenting protein function to treat or prevent disease.

Our MRT platform is potentially broadly applicable across multiple diseases in which the production of a desirable protein can have a therapeutic effect. We have observed successful production of desired proteins through multiple routes of administration in preclinical studies, which may allow us to develop MRT product candidates for the treatment of a wide range of rare and non-rare diseases, including including diseases that affect the lung, liver, eye, central nervous system and lymphatic system. MRT may also be applied to produce therapeutic antibodies and vaccines in areas such as infectious disease and oncology.



## Potential Advantages of mRNA Therapy

- Restore gene expression without entering the cell nucleus or changing the genome
- Enable the treatment of diseases that were previously undruggable by using the cell's own machinery to produce natural and fully functional proteins
- Exhibit drug-like properties that are familiar to health care providers, including the potential to repeat and adjust dosing in a chronic setting
- Permit rapid development from target gene selection to product candidate