# Synspira Therapeutics Receives Orphan Designation for SNSP113 for the Treatment of Cystic Fibrosis in the European Union

***-- Synspira received U.S. Food and Drug Administration (FDA) Orphan Drug Designation for SNSP113 in October 2018 --***

FRAMINGHAM, Mass., April 10, 2019 -- Synspira Therapeutics today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) has granted Orphan Drug Designation to the Company's lead product, SNSP113, a first-in-class glycopolymer, for the treatment of people with cystic fibrosis (CF). SNSP113 was granted Orphan Designation for the treatment of CF by the U.S. Food and Drug Administration (FDA) in October 2018. SNSP113 targets the key drivers of pulmonary decline in cystic fibrosis and other progressive pulmonary diseases, including life-threatening pulmonary infections.

"With orphan designation in both the U.S. and EU, we have achieved an important milestone in our global regulatory strategy and are committed to advancing SNSP113 as a novel approach to treating this debilitating disease," said Robert Gallotto, President and Chief Executive Officer, Synspira Therapeutics. "We believe that SNSP113 has the potential to be the first broad spectrum therapy to improve pulmonary function in patients with CF and look forward to working with the EMA as we progress the program."

Synspira is planning to initiate a study in cystic fibrosis patients with SNSP113 in 2019, a trial that is supported in part by a development award from the Cystic Fibrosis Foundation Therapeutics, Inc.

The EMA grants Orphan Drug Designation to products that are intended to treat life-threatening or chronically debilitating rare diseases, where prevalence of the condition is less than 5 in 10,000 people in the EU, and where the product has the potential to be of significant benefit. Orphan Designation provides potential incentives, including protocol assistance, reduced EU regulatory fees, potential funding for clinical trials and access to the centralized authorization procedure. If approved in the EU to treat CF, SNSP113 will be eligible for 10 years of market exclusivity, as well as an additional two years of market exclusivity following completion of a pediatric investigation plan (PIP).

***About Cystic Fibrosis***

Cystic Fibrosis (CF) is a chronic, life-threatening, progressive disease affecting approximately 75,000 patients in Europe and the U.S. People with CF have persistent lung infection, airway blockage due to mucus and biofilm build up, and chronic inflammation, thereby limiting their ability to breathe over time. In the pancreas, the mucus prevents the release of digestive enzymes that allow the body to break down food and absorb vital nutrients. In the gastrointestinal tract, they also have mucus accumulation, bacterial overgrowth, and inflammation. CF results in destruction of lung tissue, pancreatic insufficiency, CF-related diabetes, malabsorption, malnutrition, growth retardation, and liver disease, including cirrhosis.

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**About SNSP113**
The company's lead product, SNSP113, is a first-in-class inhaled glycochemistry-based therapeutic with a broad spectrum mechanism of action intended to target the underlying cascade of events that lead to progressive pulmonary disease or other life-threatening pulmonary conditions, such as pulmonary infections with nontuberculous *Mycobacteria* (NTM), *Burkholderia cepacia* complex (BCC), *Pseudomonas aeruginosa* or methicillin-resistant *Staphylococcus aureus* (MRSA). SNSP113 is designed to normalize mucin viscosity and improve mucus transport to increase airway clearance. SNSP113 disrupts the cohesion of bacterial biofilms and interacts with the cell walls of invading bacteria to increase their permeability, reduce their viability and potentiate the efficacy of antibiotics. These actions of SNSP113 lead to a reduction in the inflammatory cascade of neutrophils that can lead to pulmonary damage and fibrosis. Progressive pulmonary disease leads to overwhelming symptoms, impacts quality of life (QoL) and results in debilitating progressive lung decline.

**About Synspira**
Synspira Therapeutics is a clinical-stage biopharmaceutical company dedicated to significantly improving the lives of people with cystic fibrosis and other rare diseases, such as primary ciliary dyskinesia and bronchiectasis, where there is a high unmet treatment need. Synspira is a privately held company headquartered in Framingham, MA. The company's lead product, SNSP113, is a first-in-class inhaled glycochemistry-based therapeutic with a broad-spectrum mechanism of action designed to target the underlying cascade of events that lead to progressive pulmonary disease or other life-threatening pulmonary conditions. At Synspira we are inspired by the patients we serve and are driven to make a difference.

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