



Sionna Therapeutics Announces IND Clearance and Phase 1 Initiation for SION-638 in Cystic Fibrosis

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– *First-in-class small molecule designed to target the NBD1 domain of the CFTR protein* –

– *First subject dosed in Phase 1 study* –

Boston, MA, December 7, 2022 — Sionna Therapeutics, a life sciences company dedicated to developing highly effective and differentiated treatments for cystic fibrosis (CF), today announced the clearance of its Investigational New Drug application (IND) from the U.S. Food and Drug Administration (FDA) for SION-638, a novel small molecule designed to target the first nucleotide-binding domain (NBD1) of the cystic fibrosis transmembrane conductance regulator (CFTR) protein. The first subject has been dosed in a Phase 1 study to evaluate the safety and pharmacokinetics of SION-638 in healthy volunteers.

“Despite advances in CF treatment, the unmet need is high as current therapies can only partially correct the function of the CFTR protein, which means many patients are still at risk of experiencing exacerbations, complications, and a burden on daily life,” said Mike Cloonan, President and Chief Executive Officer of Sionna Therapeutics. “We have achieved an important milestone of advancing SION-638, a first-in-class NBD1 modulator, into a Phase 1 study. We are encouraged by data from clinically predictive CF models demonstrating the potential to normalize folding, maturation, and function of the CFTR protein, and we look forward to evaluating our ability to drive improved outcomes for people with CF in clinical trials.”

“While people with CF are living longer and more fulfilling lives, many continue to need ancillary medications and are at increased risk for exacerbations and other serious complications,” said Charlotte McKee, MD, Chief Medical Officer of Sionna Therapeutics. “We have not yet achieved the full promise of CFTR modulators, and it is important to continue advancing research that may lead to additional treatment options and further improvements in CFTR function in the years to come.”

CF is caused by genetic mutations in the CFTR protein, an epithelial ion channel that is essential for producing healthy, freely flowing mucus in the airways, digestive system, and other organs. The most common mutation in CFTR, $\Delta F508$, causes NBD1 to unfold at body temperature and severely impairs CFTR function. CF is a serious, potentially fatal genetic disease that leads to a buildup of mucus in the lungs and airways, impaired pancreatic function and other organ dysfunction that can have a significant and often severe impact on health and life expectancy. There are over 100,000 people living with CF around the world. It is estimated that 90 percent of people living with CF have the genetic mutation $\Delta F508$.

About Sionna Therapeutics

Sionna Therapeutics is a life sciences company dedicated to developing highly effective and differentiated treatments for cystic fibrosis (CF) by normalizing the function of CFTR, the key protein associated with disease progression in CF. Building on over a decade of extensive research on the genetic mutations associated with CF and founded in 2019, Sionna is advancing a pipeline of small molecules engineered to correct the protein defects caused by $\Delta F508$, the most common mutation that affects the CFTR protein. The company has a first-in-class portfolio of programs targeting correction of NBD1, the key and unique mechanism to enable full restoration of $\Delta F508$ -CFTR function, and complementary programs targeting ICL4 and TMD1. Sionna’s pipeline has the potential to deliver best-in-class efficacy and reach previously unachievable levels of long-term benefit for people with CF. For information about Sionna visit <https://www.sionnatx.com/>.

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