



## Sionna Therapeutics to Present Updates on Clinical Progress in Cystic Fibrosis at 42nd Annual J.P. Morgan Healthcare Conference

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– Company positioned to have multiple clinical-stage assets with the initiation of up to three Phase 1 clinical trials in 2024 evaluating highly potent Series 2 NBD1 modulators and complementary ICL4 directed modulator –

– SION-638, the Company's first NBD1 modulator, has completed Phase 1 SAD and MAD dosing and will complete Phase 1 in 1H24 –

**Boston, MA, January 3, 2024** — Sionna Therapeutics, a clinical-stage life sciences company dedicated to developing highly effective and differentiated treatments for cystic fibrosis (CF), today provided updates on its clinical development progress with novel small molecules designed to target the first nucleotide-binding domain (NBD1) of the cystic fibrosis transmembrane conductance regulator (CFTR) protein and complementary mechanisms. The company is scheduled to present on Tuesday, January 9<sup>th</sup> at 2:30 PM PST/11:30 AM EST during the 42<sup>nd</sup> Annual J.P. Morgan Healthcare Conference. The company's presentation will be posted on its [website](#) on January 9<sup>th</sup>.

"We have deep experience in CF and a sharp focus on advancing the development of novel small molecules targeting NBD1 and complementary modulators that enable the potential for full restoration of CFTR function for most people living with CF," said Mike Cloonan, President and Chief Executive Officer of Sionna. "It is a privilege to be invited to present at the J.P. Morgan Healthcare Conference again this year, which is a testament to the significant interest in new approaches to address the continued unmet need in CF. Too many patients are not able to achieve normal CFTR function, and some continue to experience tolerability issues with current treatment options. Based on extensive preclinical data, we believe our development candidates can enable dual modulator combinations with the potential to deliver significantly higher efficacy than available treatment options. This will be an exciting year for Sionna as we advance multiple additional programs into the clinic with potentially four clinical stage assets in 2024."

CF is caused by mutations in the CFTR gene, which codes for 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.

Sionna's lead candidate, SION-638, is the first-ever clinical-stage NBD1 modulator. Today the company announced it has completed Phase 1 single ascending dose (SAD) and multiple ascending dose (MAD) cohorts with SION-638. The study identified doses that were generally safe and well-tolerated, and target exposure from human bronchial epithelial (HBE) assay was achieved at all doses, with more time above target with increasing dose. In the first half of 2024, the Company expects to complete part C of the Phase 1 study, which includes a bioequivalence tablet study and a food effect study.

The company also announced plans to advance three additional clinical programs to Phase 1 in 2024, including two compounds from its second series of highly potent NBD1 stabilizers, SION-451 and SION-719, pending results from ongoing Good Laboratory Practice (GLP) toxicology studies. SION-109, which targets NBD1's interface with the ICL4 region, will start Phase 1 in the first quarter of 2024 and will be combined with an NBD1 stabilizer later in development to form a dual modulator combination.

### About Sionna Therapeutics

Sionna Therapeutics is a clinical-stage 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 directly 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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