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Anagram Therapeutics Presents Positive Data from Clinical Study in People with Cystic Fibrosis (CF) Related Exocrine Pancreatic Insufficiency at North American CF Conference

ANG003 is a Novel Non-Porcine Enzyme Replacement for Exocrine Pancreatic Insufficiency

ANG003 was Well Tolerated and Demonstrated a Statistically Significant Dose-Dependent Increase in DHA and EPA Fat Absorption

FRAMINGHAM, Mass., September 27, 2024 — Anagram Therapeutics Inc., a clinical-stage biopharmaceutical company dedicated to improving the lives of people with cystic fibrosis (CF) and other rare diseases, today presented results from a dose-ranging study of ANG003, a novel broad-spectrum orally delivered non-porcine enzyme replacement therapy, in people with CF who have exocrine pancreatic insufficiency (EPI). People with EPI do not produce enough pancreatic (digestive) enzymes to break down foods and absorb nutrients, which can lead to malnutrition, fatty acid abnormalities, profound gastrointestinal symptoms, a significant decrease in quality of life and reduced life expectancy.

“Data reported on ANG003 at the North American Cystic Fibrosis Conference exceeded our expectations, exhibiting a robust increase in DHA and EPA fatty acid absorption as well as a favorable safety and tolerability profile,” said Robert Gallotto, president and chief executive officer of Anagram. “Additionally, the dose-dependent effect observed at the higher doses underscores the potential of this novel investigational therapy to advance the treatment of EPI.”

The primary objective of this multicenter, randomized, parallel study was to evaluate the safety and dose ranging of orally administered ANG003 in adult subjects with CF-related EPI. Fifty-one (51) study participants with CF-related EPI, aged 18 or older, completed dosing at a Cystic Fibrosis Therapeutics Development Network center in the U.S. Individuals were randomized to one of four possible ANG003 dosing combinations of orally delivered lipase, protease, and amylase, administered with a high fat meal, to evaluate safety and identify recommended doses for further clinical development. The study assessed the absorption of the byproducts of digestion (fat, protein, and carbohydrates) in plasma.

ANG003 was well tolerated across all dose levels with no treatment-related serious adverse events and no treatment discontinuations due to adverse events. ANG003 significantly improved DHA and EPA absorption with the 40-80-120 mg doses ($p=0.005$, $p=<0.0001$ and $p=<0.0001$) of lipase, demonstrating a robust increase in plasma levels. There was dose dependent and statistically significant increased absorption of DHA and EPA with the 80mg and 120mg doses compared to the 20mg ($p=0.03$, $p=0.004$) and 40mg ($p=0.08$, $p=0.01$) doses. Docosahexaenoic acid (DHA) and eicosapentaenoic acid (EPA) are two of the most difficult to digest dietary fats and are known to be deficient in people with CF and EPI. DHA and EPA fatty acids in plasma and red blood cells are strongly correlated with dietary fat intake and are a biomarker of digestion and absorption. Total fatty acids (c14:c24) followed a similar pattern of absorption as DHA+EPA. The response was similar to that observed in the preclinical studies. The fatty acid biomarkers of absorption have the potential to monitor people living with EPI to understand disease-related changes in DHA, EPA, and overall fatty acid levels.

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"Many people living with currently available treatment options continue to experience gastrointestinal symptoms and disease progression despite a high pill burden that is often disruptive to their lives," said Meghana Sathe, MD, lead investigator. "These data indicate that ANG003 has the potential to offer an important treatment option for people with EPI."

Clinical trial data were presented today in four posters and two oral presentations at the 38th annual North American Cystic Fibrosis Conference.

"Based on the results of this study we look forward to collaborating with regulatory authorities and moving as quickly and judiciously as possible on behalf of people living with CF and others with EPI," continued Mr. Gallotto. "We can't express enough how grateful we are to the study participants, their families and caregivers, as well as the investigators who took part in the trial. We believe there is a significant need for non-porcine enzyme replacement treatment options such as ANG003 for this devastating condition, and to improve access."

About ANG003, Malabsorption Syndromes and Nutrient Metabolism Disorders
Anagram aims to create a new class of orally delivered enzyme replacement therapy (ERT) based on a deep knowledge of gastrointestinal disease pathology, advances in enzyme engineering and formulation technologies, with an experienced team that has previously built successful enzyme-based biotech companies. ANG003, Anagram's lead investigational product for the treatment of malabsorption and exocrine pancreatic insufficiency (EPI), is a new class of broad-spectrum digestive enzyme replacement therapy, targeting some of the most challenging diseases in infants, children, and adults. ANG003 contains lipase for fat malabsorption, protease for protein malabsorption, and amylase for carbohydrate malabsorption. ANG003 was engineered to be stable and immediately active in the gastrointestinal tract to maximize digestion.

People with EPI are currently treated with pancreatic enzyme replacement therapies (PERT) derived from porcine pancreas extract. The current PERT market is approximately $2 billion annually in the U.S. PERT treatment rarely eliminates maldigestion and in spite of the high treatment burden, requiring 15-40 capsules per day, patients still have chronic gastrointestinal symptoms. Malabsorption syndromes and nutrient metabolism disorders are a group of conditions caused by enzyme deficiencies or genetic disorders that prevent the body from properly processing or absorbing certain fats, sugars, proteins, vitamins or other key nutrients.

About Anagram Therapeutics
Anagram Therapeutics Inc. is a clinical stage biopharmaceutical company developing novel, orally delivered enzyme therapeutics for the treatment of serious diseases caused by malabsorption syndromes and nutrient metabolism disorders that prevent the body from properly processing or absorbing certain fats, sugars, proteins, vitamins or other key nutrients. The company is leveraging proprietary enzyme technologies and expertise in gastrointestinal diseases to solve complex problems and advance a pipeline of products that can make a life-changing impact for people and their families living with cystic fibrosis and other rare diseases. ANG003, Anagram's lead product for the treatment of malabsorption and exocrine pancreatic insufficiency, is a new class of broad-spectrum digestive enzyme replacement therapy in clinical trials in people with cystic fibrosis. Anagram is a privately held company headquartered in Framingham, MA. To learn more, visit www.anagramtx.com or follow us on LinkedIn and Twitter.

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Anagram Contact:
Kathryn Kilroy
Director, Operations
kkilroy@anagramtx.com
(617) 872-0335

Media Contact:
Gina Cella
Cella Communications
gcella@cellapr.com
(781) 799-3137 (mobile)