**FDA Grants Orphan Drug Designation, Fast Track Designation and Rare Pediatric Disease Designation to Parion Sciences P-1037 Inhalation Solution for the Treatment of Primary Ciliary Dyskinesia**

**Durham, NC (July 29, 2020) –** a clinical-stage pharmaceutical company focused on treatments of unmet respiratory diseases, announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation, Fast Track Designation and Rare Pediatric Disease Designation to P-1037 Inhalation Solution (P-1037 IS). P-1037 IS, a novel, epithelial sodium channel (ENaC) inhibitor in a nebulized solution is being evaluated for the potential treatment of Primary Ciliary Dyskinesia ("PCD"). PCD is a serious disease that affects approximately 1 in 15,000 people worldwide. Primary ciliary dyskinesia is a disease that encompasses many inherited genetic disorders that impair cilia function. PCD patients have impaired cilia that reduce mucus clearance from the airways resulting in lifelong respiratory disease with chronic, debilitating lung infections that can lead to respiratory failure.

"The receipt of Orphan Drug Designation, Rare Pediatric Disease Designation and Fast Track Designation all represent significant regulatory milestones for our P-1037 Inhalation Solution clinical development program in PCD, for which there are no currently approved treatments" said Paul Boucher, Chief Executive Officer of Parion. "We are excited about the results of P-1037 Inhalation Solution in our international Phase 2 CLEAN PCD clinical trial and look forward to continuing to investigate the potential of P-1037 IS to make a meaningful difference for this underserved patient population."

The FDA Orphan Drug Designation program provides orphan status to drugs and biologics that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the U.S. Orphan Drug Designation can provide the sponsor access to various development incentives, such as tax credits for qualified clinical trials expenses and waivers for certain FDA application fees. Orphan Drug Designation also provides up to seven years of market exclusivity in the U.S. after FDA approval is received.

A Rare Pediatric Disease is defined by the FDA as a serious or life-threatening rare disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the US. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval for a drug or biologic for a Rare Pediatric Disease may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

Fast Track Designation enables a regulatory process designed to get important new treatments that fulfill an unmet medical need to patients sooner. Fast Track Designation provides a

number of advantages to facilitate development and potentially expedite the approval process. A sponsor who receives Fast Track Designation may be eligible for more frequent communication and meetings with regulators, priority review of the marketing application and the potential for Accelerated or Rolling Approvals.

## About ENaC Inhibitors and P-1037

Epithelial sodium channel (ENaC) inhibitors are designed to block the sodium channels on the airway surfaces. In respiratory diseases, such as chronic obstructive pulmonary disease, asthma, cystic fibrosis and primary ciliary dyskinesia, where there is a build-up of excessively concentrated mucus, preclinical models have demonstrated that blocking ENaC hydrates the mucus on the lung surface. Hydration of airway mucus restores airway clearance and improves lung function. P-1037 is a novel, long acting ENaC Inhibitor that was well tolerated at the doses tested in multiple clinical trials in healthy volunteers and patients with muco-obstructive lung diseases, including primary ciliary dyskinesia. Further studies with P-1037 IS in PCD patients are being planned.

## About Parion Sciences

Parion Sciences is a development stage biopharmaceutical company dedicated to research, development, and commercialization of treatments to improve and extend the lives of patients with severe respiratory diseases. Parion has a diverse pipeline of pre-clinical and clinical candidates for the treatment of these diseases via distinctive mechanisms of action and approaches. Parion is at the forefront of ENaC development and is leveraging our scientific expertise in epithelial biology to expand our platforms and advance novel chemical compounds into muco-obstructive respiratory diseases such as severe asthma, cystic fibrosis, primary ciliary dyskinesia, bronchiectasis, chronic obstructive pulmonary disease and viral infections in the lung. Parion has received support and grant funding from the National Institutes of Health and the Cystic Fibrosis Foundation.

**For further information please contact:**

Paul Boucher
President & CEO
Parion Sciences, Inc.
pboucher@parion.com
(919) 313-1195