n-Lorem's Mission to Treat Nano-rare Patients Jan. 15, 2024

After more than three decades of advances, antisense (ASO) technology is a fully validated, broadly enabling genetic drug discovery technology. ASOs have been commercially approved for diseases of the eye (CMV retinitis), pediatric CNS (SMA), adult CNS (ALS), skeletal muscle (Duchenne's), the kidney (TTR renal disease), the PNS (TTR polyneuropathy) and CVS (hypercholestremia, hypertriglyceridemia) and multi-thousand patient phase three studies in CVS disease (hypertension, high triglycerides, CHF, secondary prevention) and similar efforts in CNS disease (Parkinson's, Alzheimer's) argue that it is poised to take its place in the treatment of the larger diseases. The safety profile of modern ASOs is established in approximately 500,000 patients, some treated for as long as 10 years.

The versatility, efficiency and cost effectiveness of ASO technology make it the ideal approach to treat extremely rare genetic diseases. In 2020, n-Lorem, a non-profit designed to provide ASO treatments to patients with the rarest genetic diseases (nano-rare) for free, for life by the acknowledged "father of ASO technology", Stanley T. Crooke, M.D., Ph.D. These patients (nano-rare patients) are so rare that they are largely undiagnosed and rarely treated today and they are in desperate need. The FDA has recognized the potential value of the technology for these patients by issuing guidance that applies to only ASO technology. The FDA guidance plus the efficiency of the technology mean that n-Lorem can mount a new ASO discovery effort and provide the first dose to most patients in less than18 months and treat for life for less than $1.5 Million. Even more encouraging, n-Lorem plans to reduce the cost per patient to less than $700,000 in the near future. In four years, n-Lorem has evaluated more than 240 patients, accepted more than 100 patients into its program, filed more than 10 INDs in four divisions of the FDA and shown benefit in severely affected patients.

n-Lorem is also committed to learning maximally from each of these "unique experiments of nature" in which a single variable, a mutation, is driving enormous phenotypic changes. n-Lorem has established processes that assure that each patient and the aggregate learnings address fundamental questions about health and disease and how effective genetic treatments may alter the molecular ontogeny of health and disease, how plastic the CNS is, what developmental delays can be recovered when and similar questions in other organs. These data will be shared with the community through presentations and publications as rapidly as possible.

n-Lorem's mission provides a viable solution for a patient population that has largely gone unnoticed and underserved. As more 'individualized' medicines are discovered and developed by n-Lorem, the potential to find others who would benefit is extremely high. Furthermore, the data gained from these patients could provide valuable insights into health and disease broadly.

Sincerely,

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