Sarepta Therapeutics Statement on ICER Draft Evidence Report for Treatments for Duchenne Muscular Dystrophy

CAMBRIDGE, Mass., May 23, 2019 -- Sarepta Therapeutics, Inc., the leader in precision genetic medicine for rare diseases, released the following statement on the report from the Institute for Clinical and Economic Review ("ICER") on treatments for Duchenne muscular dystrophy:

Sarepta is committed to appropriately pricing therapeutics so that they are cost effective and believes that, done properly, evaluations such as those conducted by ICER could serve a valuable purpose. With that said, however, ICER's approach is fatally flawed as it relates to rare and genetic disease for a number of reasons. As a result, we have chosen not to participate in reviews by ICER until it adapts its model to address the inherent limitations and biases that compromise its evaluations of therapies intended to treat patients with serious, rare diseases.

First, ICER's model is unfit to evaluate rare disease populations in a manner that would encourage innovation to bring profound treatments to patients living with, and far too often dying from, rare disease. There are 7,000 rare diseases, most of which are genetically based, and today only about 5% of those diseases have any treatment available. Great human suffering and death results from these rare diseases. Half of all those who suffer from rare disease are children. Rare disease is responsible for a third of all deaths in the first year of life and a third of children with rare diseases will not reach their fifth birthday.

After decades of extraordinary scientific advancement, biomedical innovation is bringing treatments to rare disease patients and their families who have been without any hope for far too long. ICER's model stands to halt these advancements. Consider that prior ICER evaluations have concluded that for diseases such as spinal muscular atrophy – a death sentence for those children who have it; cystic fibrosis -- a disease that robs one of the ability to breathe and then of life, and hereditary transthyretin amyloidosis – a devastating and life-limiting rare disease, proposed therapies are only cost effective if offered at discounts upwards of 90%. That is not a typographical error.

To the extent ICER's evaluations are taken seriously, no company would be able to attract investment to fund the development and manufacture of treatments for these rare diseases. Through its conclusions, ICER sends a clear message to innovators that developing rare disease therapeutics is not worth the effort,

and to patients – often children who are dying with no other treatment options – that their lives are not worth the investment.

Second, the ICER model is unequipped to accommodate the FDA accelerated approval process for new therapy approvals. Nearly 30 years ago, the FDA created an accelerated approval pathway to ensure faster approval of safe and effective drugs for serious conditions that fill an unmet medical need. The pathway relies on surrogate endpoints that are reasonably likely to lead to clinical benefit and ensure that life changing therapies for very serious diseases are sped to the medical community to ensure that patients do not suffer and die while longer clinical trials are conducted. As a reminder, this is the pathway that spurred tremendous advancements in treatments for HIV and cancer. The ICER model attempts to negate that pathway by failing to properly align with the goals of, and accepting the evidence set that supports, accelerated approval.

Third, if the goal is to support a cost effectiveness approach that promotes true innovation while acting as a watchdog for waste in the system, ICER is failing. ICER has focused nearly all its reports on evaluating and generally undermining innovative new therapies, often for rare disease. The bulk of pharmaceutical expense lies in legacy therapies, and much of the waste in the system lies in old, high-volume drugs with price increases that have historically exceeded increases in the Consumer Price Index (CPI). ICER spends little time doing the difficult but important work of evaluating waste in the large segment of healthcare spend, instead choosing to evaluate innovative new therapies that may garner headlines but do little to relieve non-innovative expense from the system.

Sarepta's mission is to find new treatments and cures for those suffering from genetic disorders and we remain focused on bringing those advances to patients as quickly as possible. We will continue to work directly with clinicians, payers, and other stakeholders to inform treatment and coverage decisions, and identify real and meaningful solutions to the challenges of patient access and sustainable innovation. And if and when ICER adapts its model to thoughtfully support investment in therapies for rare disease and the FDA's accelerated approval of those therapies, we stand ready to work with ICER as well.

About Sarepta Therapeutics

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 5 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing.

Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit www.sarepta.com.

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