Sarepta Therapeutics Statement on April 26, 2021 ICER Commentary Regarding the Accelerated Approval Pathway

CAMBRIDGE, Mass., April 26, 2021 – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today issued the following statement in response to the Institute for Clinical and Economic Review's (ICER) recent opinion piece: *Strengthening the Accelerated Approval Pathway***.**

FDA's accelerated approval pathway has brought earlier access to transformative therapies and helped countless patients since its creation in 1992, serving a profound role in driving innovation needed to turn great scientific ideas into treatment successes for patients with serious or life-threatening diseases – patients who, were it not for accelerated approval, would be left behind with zero treatment options. The accelerated approval pathway must be preserved to deliver the benefits of scientific advancement to patients as safely and as quickly as possible.

Sarepta agrees with ICER on the point that "draconian access restrictions should not be the way that the health system seeks to find the balance between uncertainty, cost, and the incentives needed for future innovation." However, ICER gets it wrong in failing to recognize that many of its policy proposals would do just that: result in draconian access restrictions and fail to strike any balance between early access, uncertainty, cost, and innovation. The approaches advocated by ICER in this commentary are not grounded in current, tangible experience with the accelerated approval pathway and would largely dismantle this critical mechanism by driving investment away from developing innovations for patients with otherwise intractable diseases and high unmet need, and, worst of all, harming patients – the exact opposite of what Congress and FDA intended years ago in establishing this critical pathway.

Similar to ICER's rigid, discriminatory health technology assessment framework, its policy proposals devalue accelerated approval drugs, send a message to innovators that developing new rare disease treatments are not worth the effort, and tell patients that their lives are not worth the investment. Proposals that serve as disincentives and block the development of life-changing treatments are never an ethical option to save money on healthcare. There can be no balance if innovation and access are removed from the equation.

Finally, in holding up eteplirsen as an example, ICER misrepresents the available science on the benefits of dystrophin and ignores the body of evidence that Sarepta and others have published and presented on since the approval of eteplirsen in 2016. Since approval, Sarepta has supported more than a dozen publications and presentations in the literature or through appropriate scientific venues, and the Company continues to work diligently to complete studies to support post-marketing regulatory requirements for all its FDA-approved treatments, as well as actively collect real world evidence.

Patients suffering from difficult and tragic diseases deserve a chance to be treated based on cutting-edge science. Sarepta continues to work directly with clinicians, patients, regulators, payers, and other stakeholders to bring this science to patients as quickly and responsibly as possible and identify real and meaningful solutions – including exploring novel payment models – to appropriately balance access, uncertainty and cost, without sacrificing patients in the process.

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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