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MassBio
MASSACHUSETTS BIOTECHNOLOGY COUNCIL

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On behalf of the Massachusetts Biotechnology Council (MassBio), an association of more than 650 biotechnology companies, universities, academic institutions and others in the Commonwealth of Massachusetts dedicated to advancing cutting edge research, I applaud the introduction of a Targeted Drugs for Rare Diseases bill.

This bill will allow companies researching and developing genetically-targeted and protein variant-targeted technologies to fully utilize those technologies to develop new therapies for serious rare diseases for patients so desperately in need.

We fully support clarifying the FDA's current discretionary authority to rely on extrapolation of data from a sponsor's previously approved products utilizing the same or similar gene or protein variant-targeted technology in reviewing and approving applications for accelerated approval of new drugs for serious or life threatening rare diseases. Such clarity will encourage companies to invest in broadly applicable technologies that harness important advances in human genetics and enable more efficient and more rapid drug development.

As you know, most rare diseases affect very small patient populations and can often can be fragmented creating even smaller subpopulations – making it more challenging, if not impossible, to conduct traditional clinical trials needed to support FDA approval. Precision medicine, and using a targeted approach to address diseases at the genetic level, holds much promise in addressing hard-to-treat rare diseases and finding new treatments and cures.

Modern gene and protein variant-targeted technologies can be adapted to develop drugs to address multiple rare disease subtypes or patient subpopulations. When the underlying platform chemistry has been demonstrated to be safe and effective based on an initial FDA approval, it is reasonable to pursue a streamlined regulatory approach to speed patient access to a subsequent therapy based on the same or similar technology that targets a different mutation in the same gene, or a different but related clinical manifestation of a disease gene, for treatment of a serious, rare condition. This approach could reduce drug development and approval times by years, and make clear the availability of a feasible approval pathway in fragmented rare diseases, in each case expediting access to new treatments for patients with urgent unmet medical needs.

We look forward to working with you to refine the language of the bill as it progresses through the legislative process. Thank you for your efforts and leadership in addressing this issue for the benefit of rare disease patients around the world.

Sincerely,

RK Coughlin

Robert K. Coughlin
President & CEO