

HR 3737, Unlocking Lifesaving Treatments for Rare-Diseases Act of 2012

Fact Sheet

We have compiled this fact sheet to help clarify the specifics of the UTLRA Act

Rare diseases need improved access to the FDA's Accelerated Approval Process. In the first 16 years of Accelerated Approval, only one drug to treat a rare genetic disease was given access to the pathway. In fact, the FDA's own [Rare Disease Report](#) cited the need to increase the science to support surrogate endpoints to improve access to the Accelerated Approval Process. The ULTRA Act will further empower the FDA to achieve this goal.

Small regulatory changes can make a huge impact. The Accelerated Approval Process changed HIV from a death sentence to a chronic manageable disease with 29 drugs approved in a 16-year period. The same innovation is possible for rare diseases through Accelerated Approval.

Three times as many rare diseases could be treated for the same investment by using the Accelerated Approval Process. [A report published in Orphanet](#) found the Accelerated Approval Process decreases the cost to develop a rare disease treatment by a mean of 62%.

The ULTRA Act levels the playing field for treatments for rare diseases. The ULTRA Act provides the FDA with the tools necessary to allow for alternative qualification criteria for the rarest diseases that could never have prior clinical data. Treatments for drugs that treat larger populations have enough patients to produce the required data and do not need this exemption.

At the current rate of approval, it will take 150 years to treat 50% of the rare disorders affecting small populations in the database. Over the last 25 years, only two or three products for rare diseases affecting small populations have been approved each year. This pace is too slow for the individuals suffering from these rare diseases and their family members. The ULTRA Act will help increase the development of treatments by creating a clear path through the FDA approval process for treatments for rare diseases, bringing more timely relief to patients and families.

The ULTRA Act enhances the FDA approval process for diseases with very low prevalence in the United States. The need to improve the review process for very rare diseases is substantiated through three analyses that show that treatments are being stalled in development due to very small patient populations and where changes to the FDA Accelerated Approval Process rules would have the most benefit. The reports are available for review below:

- [BioMedical Insights](#), a strategic and analytic health care consulting firm that specializes in new product development, marketing, and payment issues, examined the issues impacting rare disease drug development. The [draft report](#) and [slide presentation](#) show that the business model for development of treatments becomes extremely difficult for diseases with low prevalence. Only really large companies with huge cash positions are consistently able to get approvals.
- [The Potential Investment Impact of Improved Access to Accelerated Approval on the Development of Treatments for Low Prevalence Rare Diseases](#) shows how improved access to Accelerated Approval could ignite a surge in investment in biotech. This was published in the *Orphanet Journal for Rare Diseases*, and has been labeled a "highly accessed" article with more than 3,500 views in just a few months. It was also featured in the *Wall Street Journal* Health Blog.

- [Translation of rare disease research into orphan drug development: disease matters by Heemstra et al](#), showed the number of orphan designations, the degree of development pressure, and the number of drugs approved have all lagged those of larger prevalence orphan diseases.

The ULTRA Act does not open up the Orphan Drug Act. The ULTRA Act amends Section 506 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 356). It does not make any changes to the Orphan Drug Act.

The ULTRA Act does not create an “ultra-orphan” designation, nor does it legally define an ultra-rare disease. Current law already allows for a smaller subset of the rare disease community to have an extra advantage. The Humanitarian Use Devices (HUD) program established by the FDA in 1990 allows for an alternative pathway to get market approval for medical devices to help people with rare diseases or conditions under 4,000 prevalence. Here is a link about HUD on the FDA’s website: <http://1.usa.gov/Fisek>. The ULTRA Act follows the precedence established by the creation of the HUD program in providing a subset of the community a needed benefit, which in turn helps the entire rare-disease community. The ULTRA act does not currently define a cutoff number for ultra-rare.

The ULTRA Act does not create a new or separate FDA approval process. The Act allows treatments for rare diseases to use the current Accelerated Approval pathway. It does not create any additional major costs or burdens on the FDA except to write one guidance on qualification of surrogates for ultra-rare diseases; ULTRA empowers them to use the best science available.

The ULTRA Act requires that treatments must be safe and effective. ULTRA does not allow a product to get approved without a clinical trial. The FDA must still require a clinical trial of the drug in order to approve it. The Act only says the FDA cannot require independent clinical data in diseases that have never been studied before in order to qualify the surrogate endpoint that the sponsor can use to conduct a clinical trial. Once a surrogate endpoint is accepted, the developer then must obtain clinical data with the surrogate.

The ULTRA Act helps the entire rare disease community. The proposed legislation will help unlock existing science and spur the development of life saving treatments for millions of patients. The strongest case for giving greater weight to basic science research rests with the work being done on rare diseases. As the FDA gains experience in qualifying surrogate endpoints using basic science and other nonclinical data, we expect that the FDA will begin placing greater weight on such evidence, when appropriate, for diseases with larger patient populations.

If you have questions about the legislation or would like to provide feedback, please send comments to patientadvocate@kakkis.org.