How the US encourages rare disease drug development

Biomedical innovation does not always align with the expectations of commercial markets. Treatments for health conditions of significant global health importance – but affecting smaller and/or neglected populations – often do not generate sufficient revenue to support product development. This is particularly true for those with limited economic resources. As a result, research and development of potentially life-saving innovations may be constrained by insufficient commercial incentives and the difficulty of recovering costs associated with getting to market.

The US National Institutes of Health (NIH) has a long-standing commitment to advancing global health. It addresses diseases affecting millions of patients as well as orphan, paediatric, rare and neglected diseases affecting smaller populations. Research conducted through the NIH's Intramural Research Program may result in technologies with commercialisation potential to address a wide range of diseases and patient populations. The NIH cannot create spin-out companies to commercialise technologies arising from its intramural (internal) research. Therefore, it must establish a framework that effectively communicates the commercial value and development potential of its technologies to prospective partners.

The NIH is not directly involved in business decisions following the execution of a licensing agreement with an external partner. Still, it can help prospective partners better understand the development landscape for their technologies. Identifying and communicating strategies that may help offset the costs and risks associated with product development can increase the attractiveness of promising technologies. Ultimately, the goal is to facilitate their translation from the laboratory to the marketplace and, most importantly, to patients efficiently.

As a partner federal agency, the US Food and Drug Administration (FDA) has historically established regulatory incentives to encourage the development, approval and availability of life-changing therapies. One such incentive is the Priority Review Voucher (PRV) programme. It directly benefits the development of therapies for rare and neglected diseases. The PRV programme has allowed entities that obtain FDA approval for qualifying products to receive a transferable voucher that can be used to obtain priority review for a different product. The voucher may be applied to a potentially high-value therapy with greater commercial prospects or transferred to another company through a sale. The resulting economic value can help offset the financial risks associated with development and commercialisation.

To be eligible for a PRV, a product must meet several specific requirements. The company must request and receive Priority Review designation from the FDA. To qualify for Priority Review, the technology must be intended to treat, prevent or diagnose a serious condition. When Priority Review is granted, the FDA aims to take action on the application within six months, compared with 10 months for a standard review. In addition, the product must also fall within one of the eligible PRV programmes, such as those addressing certain tropical diseases, material threat medical countermeasures or rare paediatric diseases. The company may be awarded a PRV if an eligible product subsequently receives FDA approval and satisfies the applicable statutory requirements. The recent approval of Rasonque (daraxonrasib) for pancreatic cancer is an example of a priority product.

Another example is the approval of a vaccine based on noninfectious virus-like particles (VLPs) to prevent disease caused by chikungunya virus. Chikungunya is a mosquito-borne viral disease that is more prevalent in tropical and subtropical regions. It typically causes fever and severe joint pain, with symptoms that may persist for months or even years in some patients.

NIH-supported Phase 1 and early Phase 2 clinical trials demonstrated a favourable safety and tolerability profile to support further development[1,2]. NIH subsequently out-licensed the technology to an industry partner for additional clinical trials and commercial development. Because no approved chikungunya vaccine was available, Bavarian Nordic A/S, the final licensee, requested a Priority Review designation when submitting the prospective therapy for FDA approval. The product also qualified for a PRV because chikungunya is included among the eligible tropical diseases.

In February 2025, the FDA approved the NIH-licensed vaccine, VIMKUNYA, for the prevention of disease caused by chikungunya virus in individuals 12 years of age and older. Upon approval, Bavarian Nordic received a PRV, which was subsequently sold to an undisclosed company for $160 million. VIMKUNYA was launched in the US in March 2025 and subsequently in European markets. In 2025, the vaccine generated approximately $13 million in first-year sales[3]. The substantial value of the PRV relative to the vaccine's initial sales demonstrates how the PRV programme can provide significant additional economic value beyond the commercial returns of a qualifying product.

The case of VIMKUNYA and the NIH represents how technologies targeting diseases with limited commercial potential can be positioned for successful development. Therapies for rare and neglected diseases, including chikungunya, often generate limited interest among investors and commercial developers because of their relatively low anticipated market returns. Regulatory incentives such as the PRV programme can support continued development of such products and facilitate the translation of promising technologies into approved therapies for populations with unmet medical needs.

The FDA offers multiple pathways to expedite the development and regulatory review of certain products. Each programme has distinct eligibility criteria, objectives and regulatory benefits. Understanding these distinctions is important when evaluating the applicability and potential advantages of each programme. Some of the most relevant FDA expedited programmes are described below.

Priority Review Voucher (PRV) – Awarded upon FDA approval of certain qualifying products that meet the statutory requirements of an applicable PRV programme. The product itself must meet the criteria for a Priority Review designation to receive a voucher.

Priority Review – Accelerates the FDA's review of a marketing application, reducing the review goal from 10 months to six months. It does not shorten clinical development or itself create a transferable voucher. It can be used individually without the need of applying for a PRV.

Fast Track – Facilitates interactions with the FDA before and during the application review of a therapy treating a serious condition. The product may ultimately qualify for Priority Review if it meets the applicable criteria.

Breakthrough Therapy – Expedites the development of a therapy for a serious condition when preliminary clinical evidence suggests a substantial improvement over existing treatment. It includes all the benefits of Fast Track, along with more intensive FDA involvement in development.

Accelerated Approval – Allows earlier product approval for serious conditions based on surrogate or intermediate clinical endpoints that can be evaluated sooner. Products approved through this pathway are generally subject to postmarketing studies to verify clinical benefit.

Commissioner's National Priority Voucher – Launched in June 2025, this provides faster review, for a one- to two-month timeline, for selected products that address designated US national priorities.

Applications may qualify for one or more FDA expedited programmes depending on the product's eligibility. For example, in addition to receiving a PRV, the chikungunya vaccine VIMKUNYA benefited from three expedited programmes during its development and regulatory review: Fast Track, Accelerated Approval, and Priority Review. Although these programmes complemented one another, each had separate eligibility criteria and was granted independently based on the requirements.

Since the inception of the PRV programme in 2007, the FDA has awarded 95 vouchers[4]. Approximately 75% of these vouchers were associated with products targeting rare paediatric diseases. Notably, the Rare Pediatric Disease PRV Program was scheduled to sunset in 2026. However, recent legislation extended the programme through September 2029. This extension is particularly significant because paediatric populations are historically under-represented in clinical research. In part, this is because of ethical, safety and operational complexities associated with conducting paediatric clinical trials. As a result, treatment options for many rare paediatric diseases remain limited, underscoring the continued importance of incentives that encourage investment and development in this area.

The evidence regarding whether PRVs lead to new drug development is mixed. On the one hand, studies found limited evidence that PRVs independently drive new product development[5]. Some therapies that ultimately received PRVs were already well developed before the applicable PRV programme was established, making it difficult to attribute their development to the incentive. On the other hand, drug sponsors reported that the potential to receive a PRV influenced their development decisions[5]. While some sponsors described the PRV as pivotal to advancing a development programme, others identified it as one important factor among several considerations influencing investment and development decisions.

The economic value generated by PRVs, as well as the allocation of the resulting proceeds, has also been a topic of discussion. PRV sale prices have varied considerably, ranging from approximately $67 million to as much as $350 million, creating uncertainty regarding the economic value of future vouchers[5]. More importantly, the award of a PRV does not guarantee affordable or equitable access to qualifying therapy. Thus, while the programme may encourage investment and development in underserved disease areas, it does not necessarily ensure that affected populations will ultimately have access to the resulting products.

It is possible that the PRVs may be most effective as one component of a broader innovation ecosystem. For technologies with limited anticipated market returns, the possibility of receiving and monetising a PRV may improve the overall commercial value proposition – even when the voucher alone is insufficient to justify development. When combined with other development and commercialisation incentives, the PRV programme could contribute to a more favourable environment for translating promising technologies into validated and approved products.

Programmes such as the PRV can play an important role in advancing technologies from discovery and initial validation through further development and regulatory review. NIH intramural research targeting small or underserved patient populations could particularly benefit. Considering potential PRV eligibility may provide an additional source of economic value increasing the attractiveness of the invention to prospective licensees.

Technology transfer offices and regulatory authorities worldwide may also find value in similar incentive mechanisms for technologies targeting rare and neglected diseases. Regulatory incentives such as the PRV can help strengthen the economic bridge between scientific innovation and commercial development. In this sense, the value of the voucher extends beyond its monetary benefit. It can help address a fundamental question facing developers of healthcare innovations: Is this medically promising invention also worth taking the commercial risk to develop?

Ultimately, the goal should be to create an ecosystem in which this question becomes less dependent on the size or purchasing power of the affected patient population. Continued development of regulatory, financial, and commercialisation incentives can help align scientific opportunity, commercial participation, and public health need. By reducing the economic barriers that prevent promising technologies from reaching patients, these efforts can contribute to bridging the gap between unmet health needs and access to promising medical innovations.

References 1. Chang LJ, et al. *Lancet*. 2014; 384:2046–2052. doi:10.1016/S0140-6736(14)61185-5. 2. Chen GL, et al. *JAMA*. 2020; 323:1369–1377. doi:10.1001/jama.2020.2477. 3. Bavarian Nordic. Annual Report 2025. 2026. 4. Ridley DB. Priority Review Vouchers: Value. Duke University, Fuqua School of Business. Accessed August 18, 2026. 5. U.S. Government Accountability Office. *Drug Development: FDA's Priority Review Voucher Programs*. GAO-20-251. 2020.

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