RDLA Policy Primer
Prescription Drug User Fee Act (PDUFA)

Background: The Prescription Drug User Fee Act (PDUFA) was initially passed by Congress in 1992. Before PDUFA, more than 70 percent of new medications were first approved outside of the U.S., and, on average, it took 27 months to review a new drug application. During the HIV/AIDS crisis, advocates activated and demanded policy solutions to shorten product review times. As a result of strong patient community advocacy, Congress, the FDA, and the pharmaceutical industry worked together to create PDUFA.

What Did PDUFA Establish?

To shorten review times and improve the process used to review new drugs, the Food and Drug Administration (FDA) needed a large infusion of new resources. PDUFA facilitated the creation of these resources by allowing the FDA to collect 'user fees' from drug manufacturers when they submit an application to the FDA for a new drug or biologic. User fees are also paid annually for each approved product a company has on the market. In exchange for the new fees, the FDA set target review timelines, 10 months for standard review, and 6 months for products that qualify for priority review. PDUFA also established several exemptions from the user fee requirement, including for all orphan designated products.

PDUFA Reauthorization Process

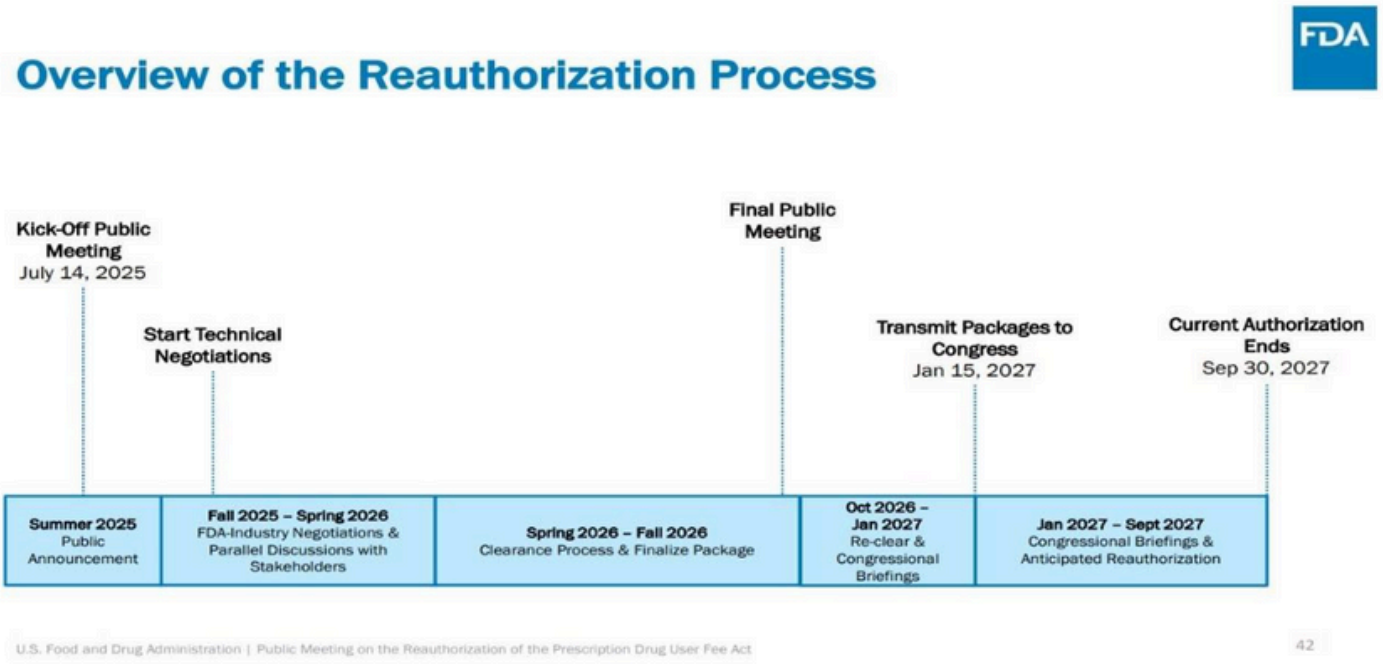
The historical timeline:

- PDUFA authorized the user fee program for an initial 5-year period, requiring a new law to be passed every 5- years since the initial 1992 law (PDUFA I) was signed.
- PDUFA has been reauthorized six times since 1992: in 1997, 2002, 2007, 2012, 2017, and 2022.
- The next reauthorization, also known as PDUFA VIII, must be passed by Congress by September 30, 2027.

The PDUFA reauthorization process:

- Before Congress gets involved, representatives from the FDA and members of the pharmaceutical industry conduct negotiations to discuss what additional actions and programs the FDA will undertake in exchange for the user fees.

- The process must start with a call for "public input on the reauthorization" and a public meeting with a 30-day comment period — all of which must take place before FDA can begin its negotiations with industry.
- The FDA is also required to hold discussions with representatives of patient and consumer advocacy groups at least once a month while negotiations proceed. The negotiations between the FDA and industry result in a "Goals Letter" or "Commitment Letter" that outlines the performance goals and activities, including pilot programs, that the FDA will undertake in exchange for the fees.
- Congress then votes to approve the "Goals Letter", usually as part of a larger legislative package, and to set the user fee funding amounts and other fees paid to FDA for the next 5 years.
- Since Congress considers PDUFA reauthorization 'must pass' legislation, they typically include several other policies that focus on enhancing how new treatments are developed and reviewed.



Rare Disease Priorities Included in Past PDUFA Reauthorizations

PDUFA V (2012)

- Established patient-focused drug development (PFDD) movement
- Clarified that the Accelerated Approval pathway should be applied in rare diseases
- Pediatric Priority Review Voucher program expanded to rare diseases
- Benefit-Risk Framework established
- Established a new breakthrough status for emerging therapies.

PDUFA VI (2017)

- Expanded the momentum of the PFDD movement
- Use of real-world evidence for regulatory decision-making
- Dedicated process to improve use of biomarkers as surrogate endpoints in drug development
- Clinical Trial Innovation

PDUFA VII (2022)

- Created the Rare Disease Endpoint Advancement pilot to facilitate the creation of novel endpoints for rare disease drug development;
- Aimed to shorten the review process for new therapy submissions and ensure earlier access to therapies through the Split Real Time Application Review (STAR);
- Sought to improve the quality and use of real world evidence (RWE)-based approaches to support improved labeling and post-approval study requirements through the Advancing Real World Evidence Program; and
- Mandated two new studies about the regulatory process for reviewing rare disease therapies.

Future PDUFA Policy Considerations:

PDUFA VIII will need to be reauthorized by September 2027. PDUFA VIII is an opportunity for the rare disease community to further improve the regulatory review process for rare diseases at FDA, including advancing the use of patient experience data, strengthening the FDA's rare disease infrastructure, advancing pilot programs, and innovations in post-market data collection.

Resources:

- EveryLife Foundation Comments on the Reauthorization of PDUFA VIII
- Rare Disease Congressional Caucus Briefing Recording (12/4/25)
- RDLA Monthly Webinar (12/2/25)
- FDA's User Fee Website