Patient Advocacy Community Praises FDA Patient Engagement Goals

National Health Council Applauds Provisions of PDUFA Agreement

Washington, DC (July 15, 2016) – The National Health Council (NHC) praised the Food and Drug Administration (FDA) for including provisions in its performance goals letter for the reauthorization of the Prescription Drug User Fee Act (PDUFA) that will strengthen patient involvement in the creation and approval of new treatments.

NHC Chief Executive Officer Marc Boutin praised the PDUFA agreement as a huge step forward for patients and the FDA:

“This agreement contains provisions that will over time significantly improve the lives of people with chronic diseases and disabilities and their families. These efforts will result in a giant step forward for the FDA’s Patient Focused Drug Development program, which was created by the last reauthorization of PDUFA. The Agency has strengthened its commitment to making patient engagement an integral aspect of drug development, and we look forward to working with them to make their goals a reality. Other provisions included in the agreement, such as increased emphasis on biomarkers and surrogate endpoints, advancing development of drugs for rare diseases, streamlining combination product reviews, studying the feasibility of incorporating real-world evidence provided from clinical settings, and improving FDA hiring and retention practices, will lead to a more efficient FDA that is better equipped to review 21st century treatments in a timely fashion.”

The letter now goes to Congress for its review and approval. Provisions in the letter supported by the NHC include:

- **Patient Engagement**: FDA commits to creating a series of guidances for industry on incorporating the patient perspective into their research and development processes. These guidances will ensure that patient engagement becomes an integral part of industry’s research and development processes. FDA also commits to updating its Benefit-Risk Implementation Plan and draft guidance on benefit-risk assessments.

- **Biomarkers and Surrogate Endpoints**: FDA will hold a public meeting on biomarkers to inform the creation of guidance. They will also increase staff capacity to qualify biomarkers and create a public website to inform the public on which biomarkers have been approved and the progress of those undergoing qualification. FDA also commits to holding early consultation meetings with industry to discuss the feasibility of new surrogate endpoints.

- **Rare Diseases**: FDA will advance the development of products for rare diseases by integrating staff of the Rare Disease Program (RDP) into product review teams. RDP staff will also train product reviewers on the challenges associated with rare disease product applications and promote best practices of rare disease product review.
• **Real World Evidence (RWE):** FDA will hold one or more public workshop(s) to discuss the use of RWE in clinical development. The workshop(s) will inform the Agency as it initiates activities, such as conducting pilot studies and crafting guidance for the appropriate use of RWE.

• **Combination Product Review:** FDA will streamline the review of products that require oversight of multiple regulatory offices and increase staff capacity of the Office of Combination Products. This will include creating Manuals of Policies and Procedures and Standard Operating Policy and Procedures, as well as creating transparency on key contacts throughout the FDA centers during combination product reviews.

• **FDA Hiring and Retention:** FDA will address key gaps in staffing. The Agency will continually study and address recruiting and retention policies to ensure it is staffed by sufficient scientific personnel.

As a response to the tireless advocacy from the HIV/AIDS community, PDUFA was created by Congress in 1992. The Act must be reauthorized every five years. PDUFA gives FDA authority to collect fees from companies that produce certain human drug and biological products. These fees play an important role in expediting the drug approval process.

**For More Information:**

- The Prescription Drug User Fee Act: [http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/default.htm](http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/default.htm)

**About the National Health Council:**

Founded in 1920, the NHC is the only organization that brings together all segments of the health community to provide a united voice for the more than 133 million people with chronic diseases and disabilities and their family caregivers. Made up of more than 100 national health-related organizations and businesses, the NHC's core membership includes the nation’s leading patient advocacy organizations, which control its governance and policy-making process. Other members include professional and membership associations, nonprofit organizations with an interest in health, and representatives from the pharmaceutical, generic drug, health insurance, medical device, and biotechnology industries. For more information about the NHC, visit [www.nationalhealthcouncil.org](http://www.nationalhealthcouncil.org).

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