Cosponsor the Scientific External Process for Educated Review of Therapeutics (EXPERT) Act (H.R.1532/S.822)

The Scientific EXPERT Act formalizes the Externally-Led Scientific-Focused Drug Development (EL-SFDD) meeting initiative at the Food and Drug Administration (FDA).

Current Challenges to the Evaluation of Rare Disease Therapies:

- Over 90% of rare diseases do not have a FDA-approved treatment and there are many challenges and hurdles facing rare disease treatments in the approval process.
- Evaluating therapies to treat small and, in some cases, very small populations as low as a single patient requires highly specialized expertise combined with a consistent approach to applying the regulatory flexibility tools authorized by Congress.
- Therapies in development for rare diseases face unique complexities, requiring exceptional scientific consultation and support.
- FDA reviewers often lack expertise in specific rare diseases, while rare disease experts often have conflicts of interest due to their research and involvement in clinical trials that prevent them from participating in FDA advisory committees.

How the Scientific EXPERT Act Addresses Challenges:

- Bridges the gaps by allowing scientists and FDA reviewers to share their expertise and knowledge in a product-agnostic setting without compromising the integrity of the review process.
- Provides additional rare disease expertise to regulatory experts through the EL-SFDD.
- Enables scientists and FDA reviewers to share their expertise and knowledge in a product-agnostic setting without compromising the integrity of the review process.
- Establishes formalized meeting structure for enhanced collaboration between medical experts, drug sponsors, scientific organizations, and patient advocates.
- EL-SFDD meetings will focus on distinct challenges impacting the development of rare disease treatments, identify scientific opportunities to facilitate development, discuss novel clinical trial designs, and align endpoints to address unmet medical needs for rare disease patients.

To cosponsor the Scientific EXPERT Act or for questions, please contact: Emma Sheffert (Rep. Matsui) at Emma.Sheffert@mail.house.gov, Huntley Campbell (Rep. Bilirakis) at Huntley.Campbell@mail.house.gov, Ruth McDonald (Sen. Klobuchar) at Ruth_Mcdonald@klobuchar.senate.gov, or Seth McCaughan (Sen. Wicker) at Seth_McCaughan@wicker.sentate.gov.