**Cosponsor H.R. 946, the ORPHAN Cures Act**

**Background**

* The *Orphan Drug Act of 1983* established incentives to encourage the research and development of innovative treatments for rare diseases.[[1]](#endnote-1)
* Prior to passage of the *Orphan Drug Act*, the FDA approved only 38 rare disease treatments; since then, over 880 rare disease treatments have been approved.
* To date, 95% of rare diseases still lack an FDA-approved treatment.
* In 2022, Medicare’s drug negotiation program was established, which exempted orphan drugs with FDA approval to treat exclusively one rare disease; *however, that legislation failed to exempt orphan drugs with FDA approval to treat more than one rare disease.*

**The Problem**

* Treatments developed and approved for one rare disease often prove effective against other rare diseases following additional R&D.
* Unfortunately, the current incentive structure encourages life science innovators to pursue FDA approval to treat only one rare disease and discourages them from taking the additional steps necessary to obtain approval to treat multiple rare diseases.

**The Solution**

* The ORPHAN Cures Act would extend proven incentives to encourage life science innovators to pursue and obtain FDA orphan drug approvals for multiple rare diseases.

**Supporting Groups**

International Foundation for Autoimmune & Autoinflammatory Arthritis, Alliance to Cure Cavernous Malformation, Biotechnology Innovation Organization, The Bonnell Foundation, CancerCare, Cholangiocarcinoma Foundation, Cure CMD, Cure POGZ Disorders Foundation, CureLGMD2i, Eosinophilic & Rare Disease Cooperative, Facing Hereditary Cancer Empowered, Firefly Fund, Friedreich’s Ataxia Research Alliance, Littlest Tumor Foundation, Little Hercules Foundation, Neurofibromatosis Network, Prevent Blindness, Cancer Support Community, Parkinson & Movement Disorder Alliance, Rare Access Action Project, Sick Cells, Hope for Stomach Cancer, Tigerlily Foundation, Undiagnosed Disease Network Foundation

To cosponsor this legislation, please contact Tyler Mortier in Rep. John Joyce’s office at tyler.mortier@mail.house.gov. or Jonathon Miller in Rep. Don Davis’s office at jonathon.miller@mail.house.gov.

1. o A disease that affects fewer than 200,000 people in the U.S. [↑](#endnote-ref-1)