Please Co-Sponsor HR 1223/S 1509, the OPEN ACT
Orphan Product Extensions Now ~ Accelerating Cures & Treatments

Congress should incentivize drug makers and innovators to "repurpose" already approved drugs for life-threatening rare diseases and pediatric cancers. Building on the science we already have will save lives and lower drug development costs.

The OPEN ACT is supported by 268 patient organizations and was passed in the House in July 2015 as part of the 21st Century Cures Act. House Co-Sponsors include: Reps. Bilirakis (R-FL) & Butterfield (D-NC). Senate Co-Sponsors include: Sens. Hatch (R-UT) & Menendez (D-NJ).

Issue: Despite advances made by the *Orphan Drug Act*, 95 percent of the 7,000 rare diseases still have no FDA-approved treatment. It is faster and cheaper to build on the science we already have than to start developing new drugs from scratch. But the number of people who have each rare disease is so small that there is no natural financial incentive for biopharmaceutical companies to consider repurposing already approved therapies to treat rare diseases.

Solution: Modeled on the incentive program in the *Best Pharmaceuticals for Children Act* (BPCA), the OPEN ACT offers a one-time six-month exclusivity extension to a company when they repurpose an existing therapy to treat a rare disease.

Background: Scientific literature shows that a single targeted drug is likely to have multiple therapeutic uses, because diseases "target" the body in similar ways. Repurposing drugs is faster, cheaper, and presents fewer risks than traditional drug development. The *Orphan Drug Act* has been an undeniable success, incentivizing industry to develop treatments for rare diseases which has led to numerous new lifesaving therapies. However, additional incentives, specific to repurposing, would speed up this progress, and, like the BPCA, could result in hundreds of newly approved therapies for rare diseases in the next five years.

Outcomes: The OPEN ACT would leverage the investment already made by biopharmaceutical companies, resulting in:

- A surge in biotech investment, new jobs, and grants to research universities to conduct clinical trials.
- Potentially hundreds of well tested treatments approved and on the label for rare disease patients in the next five years. Many of therapies would be priced at major market drug prices, thus bringing down the average cost of rare disease drugs.
- Fewer rare disease patients using untested and potentially ineffective drugs off–label.

To co-sponsor, please contact: Tom Power, Office of Rep. Bilirakis (R-FL): thomas.power@mail.house.gov or Saul Hernandez, Office of Rep. Butterfield (D-NC), saul.hernandez@mail.house.gov. For the Senate, contact: To co-sponsor S. 1509, or to learn more about the legislation, please contact Lauren Polous, office of Sen. Hatch (R-UT), at Lauren_Paulos@hatch.senate.gov, or Swarna Vallurupalli, office of Sen. Menendez (D-NJ), at swarna_vallurupalli@menendez.senate.gov.

268 Supporting Patient Organizations (and counting…)

(Partial list of supporters. For a complete list, see www.everylifefoundation.org/open-act)

A Kids' Brain Tumor Cure
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Adrenal Insufficiency United
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International Pemphigus and Pemphigoid Foundation (IPPF)
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