IRA THREATENS CURES FOR PATIENTS WITH RARE DISEASES

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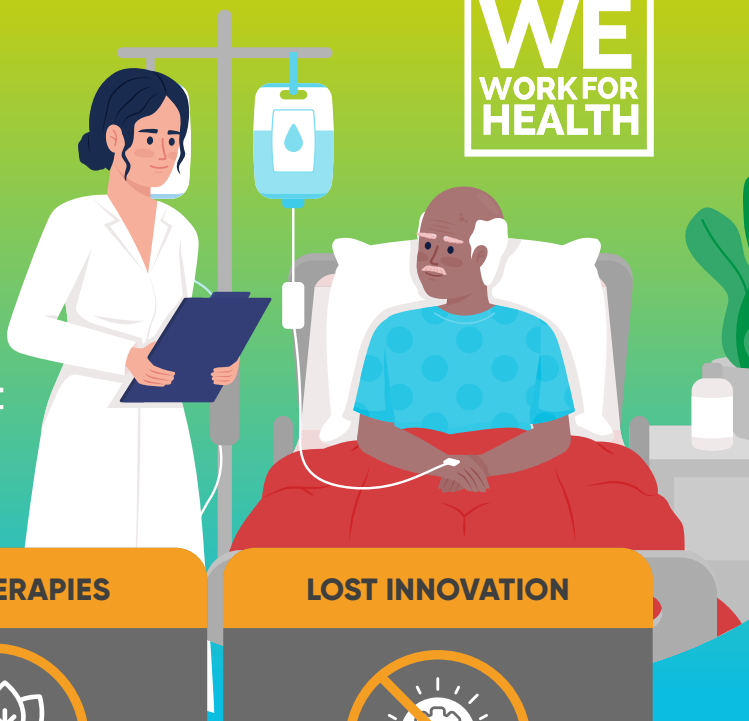

Study Shows Government-Mandated Drug Pricing Policies Threaten Development of Future Treatments for Rare Diseases

A new study by research firm Vital Transformation estimates that there would be hundreds of lost therapies if Congress expands new aggressive government-mandated drug pricing policies, including many in development to treat rare diseases, which are collectively estimated to affect 1 in 10 Americans.[1]

LOST THERAPIES

70% of all currently available medicines – treatments Americans rely on every day – would likely never have been created, including nearly a dozen different treatments for rare diseases and conditions.

LOST INNOVATION

237 fewer medicines will be FDA-approved over the next decade, especially in areas where we are desperately seeking cures – like rare diseases.

"PILL PENALTY" LOOMS OVER RARE DISEASE PATIENTS WAITING FOR CURES

The new IRA law discourages the development of medicines that come in pill or tablet form, often referred to as "small molecule medicines." Small molecule drugs have a variety of uses and modes of administration and can be scaled up to tackle a wide range of diseases. They also have an important role to play in helping advanced treatments – such as cell and gene therapies – work better.[2] In addition, small molecules may be taken orally, which gives patients with mobility issues easier access to treatment and the ability to be treated at home instead of a healthcare delivery setting.

	IRA	WH BUDGET PROPOSAL & SENATE SMART PRICES ACT
Time Limit Between FDA Approval & Mandated Price	Small-molecule drugs **9 years** Biologics **13 years**	All drugs **5 years**

Mandating prices on medical discoveries so soon after they're approved will have a chilling effect on the development of treatments for rare diseases.

1. National Institutes of Health. About GARD. Genetic and Rare Diseases Information Center. https://rarediseases.info.nih.gov/about
2. Brayshaw LL, Martinez-Fleites C, Athanasopoulos T, Southgate T, Jespers L, Herring C. The role of small molecules in cell and gene therapy. RSC Med Chem. 2020 Dec 24;12(3):330-352.

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FUTURE OF ORPHAN DRUG DEVELOPMENT DERAILED BY THE IRA

The Orphan Drug Act of 1983 has been a resounding success in encouraging the development of treatments for rare disease. Today, more than 50% of FDA approvals of new drugs annually are orphan drugs.[3] While drugs with an orphan drug designation are exempt from the IRA's price controls, receiving an indication for *any* additional disease would take away this exemption. This will discourage companies from researching new indications for their existing drugs that could help more people.

WHAT THEY'RE SAYING ABOUT INVESTMENTS IN RARE DISEASE TREATMENTS

"I think very pertinent to us in the near-term is that while the exemptions in the Inflation Reduction Act from Medicare price negotiation for a drug that has a single orphan drug indication, there are now disincentives to pursuing the approval of drugs that in additional indications." **– Yvonne Greenstreet, CEO of Alnylam Pharmaceuticals, Inc.[4]**

Small-molecule drugs are important in rare disease treatment. But the penalty means drug companies might steer research dollars away from this medicine class and towards biologics. Biologics are usually administered in a clinical setting, so it's more difficult for vulnerable groups to access them. These two provisions threaten to rob millions of rare-disease patients of future breakthroughs." **– Jenifer Ngo Waldrop – Executive Director of the Rare Disease Diversity Coalition[5]**

LEARN MORE

The new study issued by We Work For Health (WWFH) and Vital Transformation modeled the impacts on industry revenues, jobs and future research and development investments. For the full study, please visit We Work For Health.

3. U.S. Food & Drug Administration. "New Drug Therapy Approvals 2022, Advancing Health Through Innovation." January 10, 2023.
4. Alnylam Pharmaceuticals. "Q3 2022 Earnings Call Transcript". October 27, 2022.
5. Ngo Waldrop, Jenifer. Waldrop: Inflation Reduction Act undermines rare disease research. Boston Herald. February 28, 2023.