HOUSE BUDGET COMMITTEE

HEALTH CARE TASK FORCE

House Budget Health Care Task Force Chair Burgess' Statement on Rare Disease Day

WASHINGTON, D.C. — Today, House Budget Committee Health Care Task Force Chair Michael C. Burgess, M.D. released the following statement in recognition of Rare Disease Day:

> *"Today, Members of Congress are recognizing Rare Disease Day, a day where patient advocates take their critical mission to our nation's Capital to promote policies to support the millions of Americans impacted by rare diseases and encourage the development of new cures and treatments. To all the patient advocates walking the halls of Congress, thank you for coming. Your voices are needed now more than ever as Congressional Democrats and President Biden work to advance policies that run the risk of derailing progress toward curing rare diseases by disincentivizing innovative research and development.*
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> *"Democrats' so-called Inflation Reduction Act, for example, imposed government price controls on prescription drugs, and has already forced biopharmaceutical innovators to halt the development of new cures and treatments, particularly for rare diseases. The House Budget Committee's Health Care Task Force will continue sounding the alarm on the impact of Democrats' price controls on patients and engaging the Congressional Budget Office (CBO) to accurately estimate the dangerous impacts of these misguided policies and the subsequent impact to the budget."*

WORD ON THE STREET

The IRA Needs Changes to Better Support Patients with Rare Diseases (STAT): "Unless Congress acts, the IRA threatens to unravel one of America's greatest medical and public policy success stories: the explosion of new treatments for patients living with rare diseases.

[D]rugs being studied for more than one rare disease will be fair game for negotiation even before they've been FDA-approved for additional indications, **creating huge disincentives to explore uses for additional rare diseases**."

How The IRA Could Delay Pharmaceutical Launches, Reduce Indications, And Chill Evidence Generation (Health Affairs Forefront):
"That is, the incentives created by the IRA—namely, the start of the seven-year "clock" toward selection for price-setting—suggest that it may now be advantageous to wait to launch a product in the US until other indications are also ready for launch.

Such incentives could **disproportionately impact patients with rare diseases** who may have to wait for access to innovative treatments when, pre-IRA, a product would have otherwise been launched first in their disease state."

IRA's Impact on the US Biopharma Ecosystem (Vital Transformation): IRA provides a negotiation exemption for orphan drugs that treat only one rare disease. This disincentivizes investments in orphan drugs and areas of high unmet patient need as the broader indications will provide a superior return on investment, as much as $500 million over three years.

- This could result in a **reduction of FDA approvals of new orphan drugs from 34 to 14 from 2026 to 2035**.

THE BOTTOM LINE

Congressional Republicans have led the way to foster America's global leadership in biomedical research and development, giving hope to Americans for access to new treatments and cures, particularly those with rare diseases.

One of the landmark legislative achievements, the 21st Century Cures Act (P.L. 114-255) was signed into law in 2016 and included important reforms to expedite patient access to new medicines and increase patient involvement in the drug development process.

Today, the House Energy & Commerce Subcommittee on Health continued this important work, holding a hearing examining proposals to support patients with rare diseases.

The Budget Committee Health Care Task Force (HCTF), led by Rep. Michael C. Burgess, M.D. will continue engaging the nonpartisan Congressional Budget Office (CBO) to ensure accurate analysis of policies that impact patient access to new medicines, including for rare diseases.

In response to the HCTF's work, CBO recently published a blog post (A Call for New Research in the Area of New Drug Development) requesting feedback to help inform potential improvements to its drug development model and related analysis.

Policymakers need to fully understand the tradeoffs associated with proposed policies that could stifle medical innovation along with the corresponding direct impact on the federal budget, and, more importantly, American patients and families.