The Right to Try for Individualized Treatments Act

The Problem: Patients suffering from ultra-rare, life-threatening, or severely debilitating diseases often have no approved treatment options. While advances in genomics and precision medicine now make it possible to design **individualized therapies tailored to a patient's unique genetic mutation**, current regulatory pathways were not designed for treatments developed for a single patient or extremely small populations. Traditional drug development timelines can take many years, leaving patients with rare conditions without meaningful treatment options. On February 23, 2026, the U.S. Food & Drug Administration (FDA) issued draft guidance outlining a framework to support development of individualized therapies based on a "plausible mechanism" approach. While this represents an encouraging step forward, guidance alone does not provide durable patient access and can be revised or withdrawn at any time. Congressional action is needed to establish a **clear statutory pathway for patient access to individualized treatments.**

The Solution: The **Right to Try for Individualized Treatments Act** creates a responsible and patient-centered pathway allowing eligible patients to access investigational **genomics-based individualized therapies** when no approved treatment options remain. The legislation builds upon the success of the original **Right to Try Act of 2017** (P.L. 115-176) — which allows terminally ill patients to access experimental, investigational drugs that have not yet been fully approved by the FDA — while adapting it to the emerging era of precision medicine and individualized therapeutics.

Key Provisions:

- Allows patients diagnosed with a life-threatening or severely debilitating disease to access investigational individualized therapies when no approved treatments remain.
- Requires treatment to occur under physician supervision in qualified healthcare facilities meeting established federal safety and quality standards.
- Ensures institutional review board (IRB) oversight and robust informed consent protections for participating patients.
- Supports the generation of real-world clinical data that can inform future regulatory decisions and therapeutic development.
- Provides a durable statutory framework that does not depend on changing regulatory guidance or administrative priorities.

Complementing FDA's Work: The legislation does not bypass FDA authority or undermine drug approval standards. Instead, it complements FDA's ongoing work by creating a responsible pathway for individualized therapies to be administered while generating valuable clinical insights. While FDA's recent guidance focuses primarily on industry development pathways, this legislation focuses on patients who have exhausted all approved treatment options and cannot wait for traditional approval timelines.

Why Congress Must Act: Guidance documents do not create enforceable rights for patients and can change with future administrations. Only Congress can establish a durable, patient-centered statutory pathway ensuring that individuals facing life-threatening diseases have the opportunity — under physician supervision and appropriate safeguards — to pursue individualized therapies that may represent their only remaining hope. Advances in **precision medicine and genomics** are transforming healthcare. Federal policy must evolve alongside these innovations to ensure that patients can benefit from them.

Bottomline: The **Right to Try for Individualized Treatments Act** represents a forward-looking approach to modern medicine — one that promotes innovation while empowering patients and physicians to make informed treatment decisions. Congress should act to ensure that patients facing the most devastating diseases are not left waiting for regulatory timelines their conditions simply will not allow.