Ranking Member Mike Braun's Opening Statement
"Unlocking Hope: Access to Therapies for People with Rare, Progressive, and Serious Diseases"

There are over 7,000 rare, progressive, and serious diseases known to man. It is estimated that 95 percent lack treatment.

Yet in my time as a Senator, there has not been one Senate hearing to examine access to promising therapies for people with rare, progressive, and serious diseases.

This room is packed with people, patients, and caregivers that live this reality every single day. There are:

- People that watch their bodies deteriorate in real time.
- Family members who have seen their loved one go from the picture of health to knocking on death's door.
- Parents that buried their children before they'd even started school.

I want to recognize and thank each one of you here in the audience.

Chairman Casey, in response to this hearing, our Committee has received over 350 letters from Americans across 44 states sharing stories about their personal experiences with rare, progressive, or serious illnesses. I ask unanimous consent to enter these letters into the record.

A wide variety of disease groups are represented in these submissions, including ALS, DIPG- an extremely aggressive pediatric cancer, and Barth Syndrome-an ultra-rare genetic disorder that regularly results in death from heart failure before a child's fifth birthday.

Importantly, none of the diseases that our constituents have contacted us about have any form of FDA-approved treatment.

These diseases are relentless, widespread, and devastating. Most are considered a death sentence.

But they *are not* invincible, and our constituents *are not* hopeless. Patients deserve a promising pathway at the FDA.

Today, I am joined by Senators Gillibrand, Wicker, Cramer, Murkowski, Manchin, and Warnock in sponsoring the Promising Pathway Act to create a more flexible, accessible, and compassionate drug approval system at the FDA.

The Promising Pathway Act would create a rolling, real time drug approval pathway to speed access for individuals with rare, progressive, and serious diseases.

This bill does not undermine patient safety or FDA's gold standard for drug approvals in any way. Therapies developed through the pathway will be rigorously evaluated and continuously studied using real world data.

I ask unanimous consent to enter into the record a letter of support for this hearing signed by 15 individuals and organizations.

The Promising Pathway Act is a commonsense step to give FDA the flexibility it needs to serve Americans with the most trying diseases.

Our constituents continue to fight for new treatments, greater access, and full lives.

I ask my colleagues to join me in unlocking hope for these constituents.