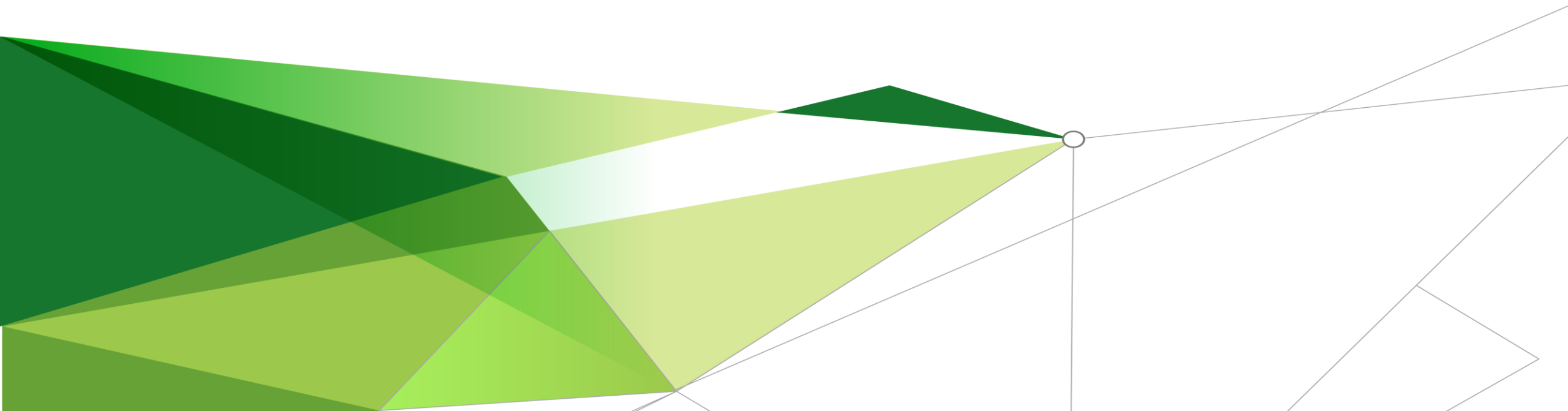


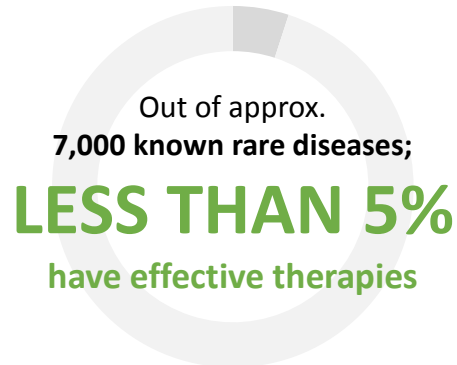
What is Rare Disease?



Rare Disease



The Rare Disease Act of 2002 defined as any disease or condition that affects **FEWER THAN 200,000 PEOPLE IN THE UNITED STATES**



Over ½ of patients with rare disease are children.

**30% OF THESE CHILDREN
WILL DIE BEFORE REACHING
THEIR 5TH BIRTHDAY**



RARE DISEASE DAY

first held in the EU and
Canada in 2008

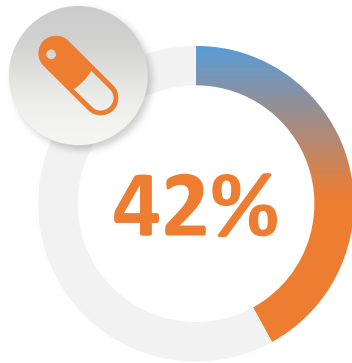


Now observed in more than
65 NATIONS;

supported by patient organizations,
the NIH, medical researchers,
hospitals and universities and
pharmaceutical companies developing
treatments for rare diseases



The Changing Landscape



Personalized medicines represent **nearly half of drugs** in the pipeline today

Tufts Center for the Study for Drug Development (CSDD)

A new product that can benefit **30,000 patients** rather than 3M requires a special kind of advocacy as it enters the marketplace



White House is requesting an initial budget of

\$215 million

for its precision medicine initiative



President Obama believes “the possibilities are boundless” and the administration’s program begins with a plan to collect genetic data on one million Americans so scientists can develop more diagnostics and therapies tailored to the characteristics of individual patients

How will all this information come together to create a complete, personalized treatment plan?