

Rare diseases are not as rare as you may think

In the EU, a rare disease affects **1 in 2000** people or fewer¹



~350,000,000

are affected worldwide.² This is more than the US population

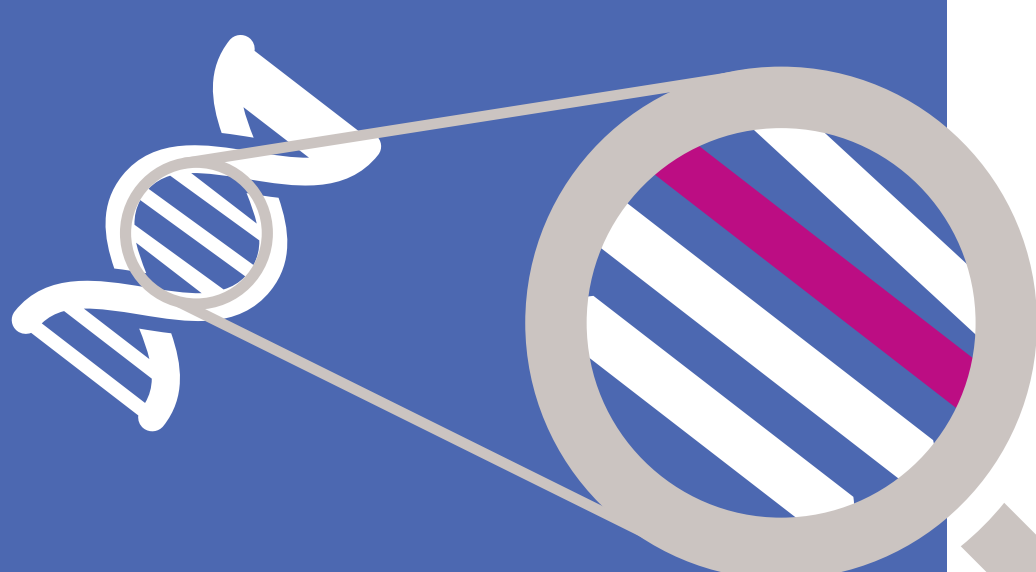


~50% of rare diseases affect children²

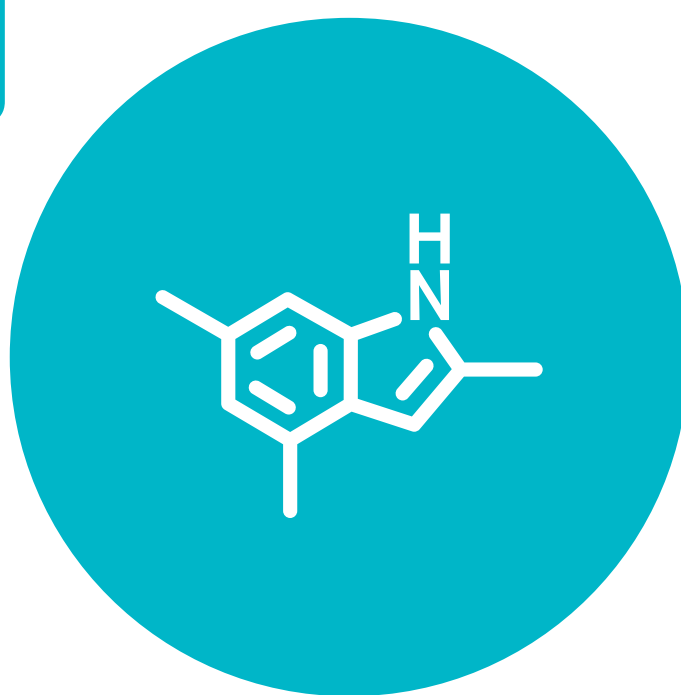


80%

of rare diseases are caused by faulty genes³

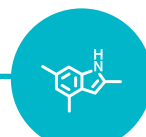


There are between **6000 – 8000** rare diseases³



~95%

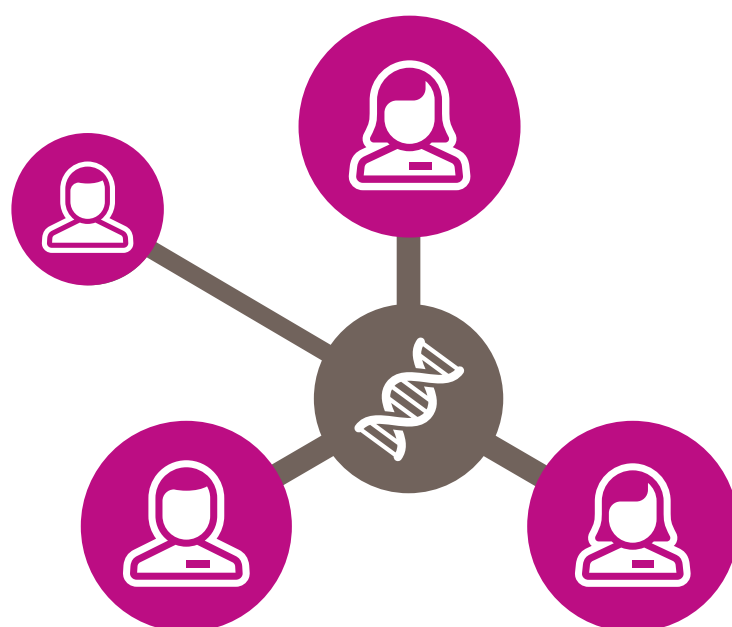
of rare diseases in the US do not have an approved treatment⁴



We have a rare disease unit that harnesses our scientific expertise, technologies, partnerships and collaborations, global reach and manufacturing capability to provide breakthrough solutions for people affected by rare diseases.

Our active research programmes cover four areas of focus:

- Rare pulmonary disease
- Rare immune disorders
- Rare neuromuscular disorders
- Rare protein synthesis disorders



See how we are helping with **#unscramblingthecode** for **#rarediseases**.

GTAACTAGGTGTC
TGCTGTCCAAGTG
TGTCA**RARE**GT
TGTCCCAAGTGAGA
TGT**DISEASES**TGTC
AGTGACAAG

Unscrambling
The code

References

¹ http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp

² <https://globalgenes.org/rare-diseases-facts-statistics/>

³ www.eurordis.org/sites/default/files/publications/Fact_Sheet_RD.pdf

⁴ phrma.org/sites/default/files/pdf/Rare_Diseases_2013.pdf