THERE ARE **7 BILLION**
people worldwide

ULTRA-ORPHAN LYSOSOMAL STORAGE DISORDERS (LSDs) AFFECT
1,000–10,000
people across the globe

LSDs ARE MULTISYSTEMIC DISEASES
presenting with a range of problems

AGE OF ONSET

INFANCY — ADULTHOOD

LIFE EXPECTANCY

FEW MONTHS — SEVERAL YEARS — ALMOST NORMAL

LAUNCHED PRODUCTS

- Gaucher
- Fabry
- Nephropathic cystinosis
- Hurler (MPS I)
- Hunter (MPS II)
- Morquio A (MPS IVA)
- Maroteaux–Lamy (MPS VI)
- Niemann-Pick Type C
- Pompe
- Ocular cystinosis

>50 Number of all LSDs

14 Number of launched products for LSDs in the US and EU

10 Number of individual LSDs treated by those products

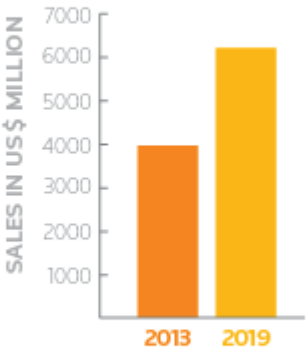
11 Number of currently untreated specific LSDs for which there are therapies with orphan status in development

The majority of launched LSD therapies are **Enzyme Replacement Therapies (ERTs)**

- ERT
- Substrate reduction therapy
- Supplement

Developing drugs for ultra-orphan LSDs proves profitable for pharmaceutical companies

Combined sales revenue of launched ERTs in 2013 and forecast for 2019



ERTs CANNOT CROSS THE BLOOD-BRAIN BARRIER to treat the neurological symptoms of LSDs

Several therapies in development aim to overcome this limitation, including gene therapy, second-generation ERTs, substrate reduction therapy and chaperone therapy

genzyme
A SANOFI COMPANY

GENZYME MARKETS AND DEVELOPS THE MAJORITY OF LSD THERAPIES, followed by Shire and BioMarin

Shire BIOMARIN

ULTRA-RARE THERAPIES CAN COMMAND ULTRA-HIGH PRICES

*The launched ERTs are amongst the most expensive therapies ever, priced at up to **$380,000 per patient per year***