Fondazione Telethon advances research on rare genetic diseases from the laboratory to patients through three connected levers: funding excellent research via independent international peer review; identifying business opportunities by valorizing discoveries (IP, orphan drug designation, start-ups and investor networks); and — crucially — de-risking, using in-house GLP, GMP, regulatory, CMC and clinical competencies to make assets safer and more attractive to partners. The Wiskott-Aldrich Syndrome (WAS) gene therapy exemplifies this model: an asset industry abandoned, which Telethon carried to approval — becoming the first non-profit to take a gene therapy from discovery to regulatory approval in both the EU and the US (Waskyra™).