High-value rare disease R&D deals of 2021

In the past decade, deals involving rare diseases have increased in size and value, supported by the growing number of opportunities based on novel therapeutic platforms.

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Dealmaking related to the development of therapies for rare diseases has accelerated significantly over the past decade, particularly in the past few years. With the help of DealForma, here we highlight annual rare disease partnership activity (Fig. 1) and high-value rare disease R&D deals signed so far this year (Table 1).

This acceleration has been supported by the clinical and commercial success of therapies for rare diseases, as well as the increasing opportunities to directly target the root cause of rare genetic diseases with novel platforms. For example, oligonucleotide-based therapies and gene therapies based on adeno-associated viral (AAV) vectors have provided major advances for rare diseases, such as spinal muscular atrophy, and pioneering products such as Spinraza (nusinersen; Biogen) and Zolgensma (onasemnogene abeparvovec; Novartis) have shown the potential for blockbuster-level sales. Products based on other platforms such as CRISPR–Cas9-mediated genome editing are in clinical trials for diseases such as sickle cell disease, and the deals in Table 1 illustrate the diversity of platforms in development for rare disease therapies.

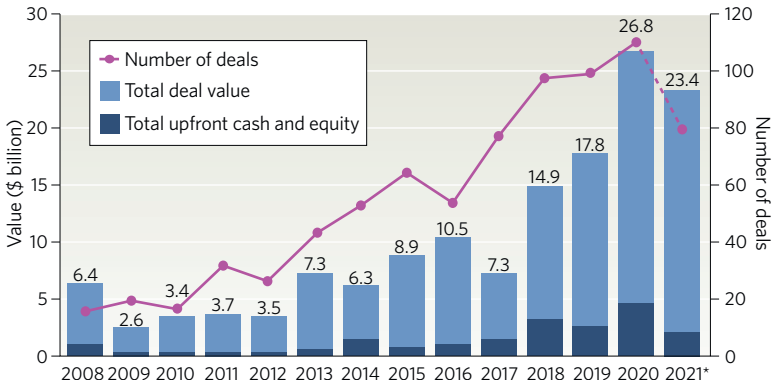

Fig. 1 | Annual rare disease R&D partnership activity. Source: DealForma database, searched for research and licensing transactions with a disclosed value for rare disease indications and early-stage deals stated to be for rare diseases in general. *Data for 2021 are up to 2 November.

Table 1 | Top ten rare disease R&D partnerships by disclosed upfront cash and equity in 2021

Companies	Date	Total deal value (upfront payment)	Deal summary
CRISPR Therapeutics, Vertex Pharmaceuticals	April 2021	$1,100 million ($900 million)	Vertex amends 2015 deal with CRISPR to expand capabilities and lead development and manufacturing of CTX001, an autologous ex vivo therapy for sickle cell disease and β-thalassemia.
Ovid Therapeutics, Takeda Pharmaceutical	March 2021	$856 million ($196 million)	Takeda buys global rights to soticlestat, a small-molecule inhibitor of cholesterol 24-hydroxylase for developmental and epileptic encephalopathies, including Dravet syndrome, from Ovid.
Syndax Pharmaceuticals, Incyte	September 2021	$602 million ($152 million)	Syndax partners with Incyte to develop and commercialize axatilimab, an anti-CSF1R monoclonal antibody for chronic graft-versus-host disease and fibrotic diseases.
Anima Biotech, Takeda Pharmaceutical	March 2021	$1,220 million ($120 million)	Takeda signs research collaboration to discover and develop novel mRNA translation modulators for neurological disorders using Anima's translation control therapeutics platform.
Ensoma, Takeda Pharmaceutical	February 2021	$1,350 million ($100 million)	Takeda licenses exclusive, worldwide rights to Ensoma's Engenious vector platform to develop therapies for up to five rare genetic diseases. Ensoma will conduct preclinical development.
Capsida Biotherapeutics, AbbVie	April 2021	$620 million ($90 million)	Capsida partners with AbbVie to develop three gene therapies for central nervous system diseases using its high-throughput adeno-associated virus engineering platform.
Argenx, Zai Lab	January 2021	$175 million ($75 million)	Zai Lab gains exclusive rights to develop and commercialize efgartigimod, an antibody fragment that targets the neonatal Fc receptor, for autoimmune diseases in Greater China from Argenx.
Atara Biotherapeutics, Bayer AG	January 2021	$670 million ($60 million)	Atara and Bayer to develop ATA2271 and ATA3271 using Atara's Epstein–Barr Virus T cell platform with CAR T technologies for non-small-cell lung cancer and malignant pleural mesothelioma.
ProQR Therapeutics, Eli Lilly	September 2021	$1,300 million ($50 million)	ProQR signs licensing deal with Eli Lilly to develop new therapies for up to five targets for genetic disorders of the liver and nervous system using ProQR's axiomer RNA editing platform.
Poseida Therapeutics, Takeda Pharmaceutical	October 2021	$3,725 million ($45 million)	Takeda partners with Poseida to develop up to eight non-viral in vivo gene therapy programs, using Poseida's piggyBac, Cas-CLOVER, biodegradable DNA and RNA nanoparticle delivery technology.

Source: DealForma database (Deals announced between 1 January – 2 November 2021)