Our list includes five public companies and five private companies.

The public companies are ranked by their 2018 revenues.

The private companies are ranked by the total capital they have raised, as disclosed in press statements.

PUBLIC COMPANY

1 Sangamo Therapeutics

2018 revenue: **$84.452 M**

Sangamo researchers have focused recently on enhancing genome editing using zinc finger nucleases (ZFNs). On July 29, a Sangamo team reported in *Nature Biotechnology* positive results from adjusting the binding affinity of the zinc finger array which recognizes DNA, and slowing the catalytic rate of the Fok1 cleavage domain. In March, another team reported in *Nature Communications* a 64-fold increase in the diversity of ZFNs available for targeting any DNA segment. Sangamo more than doubled its revenues from $36.567 M in 2017, primarily through collaborations with **Kite** (a **Gilead** company) and **Pfizer**.

PUBLIC COMPANY

2 Horizon Discovery Group

2018 revenue: **£58.7 M ($71.286 M)**

Revenue jumped 68% over 2017, which was restated to £35 million ($42.5 million) to reflect the return of £1.7 million ($2.1 million) received in error in connection with the **Dharmacon** acquisition. Horizon's topline growth reflects recent partnerships: **Celyad** is developing the first CAR T-cell therapy to use Horizon Discovery's SMARTvector™ short hairpin RNA platform. CYAD-02, a next-generation autologous, NKG2D-based candidate, is set to start a Phase I trial in early 2020. Last year, **AstraZeneca** adopted Horizon Discovery's Edit-R crRNA libraries as part of its functional genomics discovery platform.

PUBLIC COMPANY

3 Editas Medicine

2018 revenue: **$31.937 M**

R&D revenue more than doubled from $13.7 million in 2017, thanks to collaborations with Beam Therapeutics, **Juno Therapeutics** (acquired last year by **Celgene**), and especially Allergan. Editas and **Allergan** announced plans in April to begin dosing patients in the first-ever clinical trial of an in vivo CRISPR medicine. The Phase I/II Brilliance trial (NCT03872479) will assess the safety, tolerability, and efficacy of EDIT-101 for Leber congenital amaurosis 10 (LCA10). The open-label, dose-escalation study is among top priorities for Cynthia Collins, named permanent president and CEO of Editas.

PUBLIC COMPANY

4 Intellia Therapeutics

2018 revenue: **$30.434 M**

Through a collaboration launched in 2016, Intellia and **Regeneron** are partnering on Intellia-led NTLA-2001, the company's lead in vivo program for transthyretin amyloidosis—for which Intellia said August 1 it initiated IND-enabling toxicology studies and expects to submit an IND application in mid-2020. In December 2018, Intellia expanded its cell therapy collaboration with **Novartis** to include ocular stem cells—resulting in Novartis paying Intellia $10 million—and obtained expanded access to Novartis' LNP library, including rights to use the lipids for in vivo or ex vivo applications in any genome editing technology.

PUBLIC COMPANY

5 Precision BioSciences

2018 revenue: **$10.883 M**

Since completing its initial public offering on April 1, raising approximately $130.9 million in net proceeds, Precision BioSciences has begun patient dosing in a Phase I/IIa trial (NCT03666000) of PBCAR0191, the company's first gene edited allogeneic anti-CD19 CAR T-cell product candidate, being developed with **Servier**. Last month, Precision opened its Manufacturing Center for Advanced Therapeutics in Durham, NC, which the company says is the first in-house current cGMP-compliant manufacturing facility in the United States dedicated to genome-edited, off-the-shelf CAR T-cell therapies.

PRIVATE COMPANY

1 Casebia

Total capital raised: **$300 M**

A 50-50 joint venture between CRISPR Therapeutics and Bayer, Casebia was launched in December 2015 when Bayer agreed to pay CRISPR Therapeutics a minimum $300 million in R&D investment over five years, plus $35 million cash toward a minority stake in CRISPR Therapeutics. Last month at the International Society on Thrombosis and Haemostasis 2019 Congress, principal scientist Alan Brooks, PhD, highlighted research on a gene editing approach to manage production of Factor VIII for treating hemophilia A, by showing stable, titratable expression of Factor VIII in mice using CRISPR-Cas9.

PRIVATE COMPANY

2 Poseida Therapeutics

Total capital raised: **$226.5 M**

Novartis invested $75 million in equity to lead Poseida's $142 million Series C financing, completed in April. Poseida said proceeds would accelerate efforts to create gene therapy candidates based on its platform of proprietary, noninfringed gene delivery and editing technologies that could result in single-treatment cures for numerous oncologic indications and orphan genetic diseases, with an initial focus on CAR T-cell therapies. In May, Poseida received the FDA's orphan drug designation for its lead CAR T-cell candidate, P-BCMA-101, for relapsed and/or refractory multiple myeloma.

PRIVATE COMPANY

3 Beam Therapeutics

Total capital raised: **$222 M**

After launching with $87 million in Series A financing, Beam Therapeutics completed a $135 million Series B round, with proceeds intended for advancing development of the company's next-generation CRISPR technologies, expanding its pipeline of base editing programs, and extending its scientific and technical leadership. In May, Beam Therapeutics and **Bio Palette** agreed to an exclusive cross-license around each company's base editing intellectual property. Beam licenses foundational base editing intellectual property from Harvard University, the Broad Institute, MIT, and Editas Medicine across multiple platforms.

PRIVATE COMPANY

4 Synthego

Total capital raised: **$158 M**

A month before making Forbes' list of Next Billion-Dollar Startups 2019, Synthego announced some knockout news of its own, namely the launch of its Gene Knockout Kit v2. The kit is designed to guarantee a gene knockout by leveraging a novel bioinformatics-powered multi-guide strategy that employs up to three modified sgRNAs targeting the same gene. Synthego says the kit will accelerate research by saving scientists from multiple trial-and-error cycles in optimizing their CRISPR experiments. In October 2018, Synthego completed a $110-million Series C financing led by Founders Fund.

PRIVATE COMPANY

5 Inscripta

Total capital raised: **$134.5 M**

In June, Inscripta publicly presented for the first time its CRISPR-based technology, designed to enable multiplexed, trackable editing of cells at an unprecedented scale. The company has developed a family of CRISPR enzymes called MADzymes, custom nucleases for researchers and commercial partners, and a full suite of gene editing tools—instruments, reagents, and software—intended to significantly increase the speed and efficiency of multiplexed, precision gene editing. In April, Inscripta raised an additional $20 million to complete an expanded, $105.5-million Series C financing.

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