

Do you need a license to use CRISPR in drug discovery and development research? Here's what you need to know



Your connection
to CRISPR/Cas9



Michael Arciero
Vice President IP &
Commercial Development

CRISPR is fast becoming an indispensable tool in drug discovery and development, but when do you need a license to use it? Michael Arciero, VP of Intellectual Property and Commercial Development at ERS Genomics, which provides access to the foundational patents covering CRISPR/Cas9, answers some of the most common queries when it comes to deciding whether you need to license this powerful gene editing tool.

Based on the Nobel Prize winning invention of Emmanuelle Charpentier and Jennifer Doudna, CRISPR/Cas9 is an exciting gene editing technology with many research and commercial applications, including in the discovery, development and manufacture of novel therapeutics.

Perhaps less exciting, but no less important, are the legal implications of using CRISPR. As a patented technology, many uses of CRISPR for commercial applications will require licensing - including drug discovery and development.

The patent landscape for CRISPR may at first seem confusing, especially following the latest patent rulings in the US. So, when exactly do you need a license for CRISPR for your drug development research, and how do you go about getting one?

Legal note: This article applies to the use of CRISPR/Cas9 gene editing technology in the US. It does not constitute legal advice and is not exhaustive - please seek guidance from intellectual property advisors for your specific application and region.

When do I need a license to use CRISPR in drug discovery and development?

Broadly speaking, any commercial product or service that involves the use of CRISPR/Cas9 in its development or manufacturing needs a license.

This could include:

- Using CRISPR to modify CHO or yeast cells to produce a biologic drug or any other product.
- Using CRISPR to humanize animals for antibody discovery or production.
- Developing derivatives of CRISPR/Cas9.
- Creating, selling, or sharing CRISPR-derived outputs, including engineered cells, CRISPR libraries, or even data, with other organizations.
- Selling services that use CRISPR, such as generating knockout cells or animals or running CRISPR screens, to other organizations or individuals.

However, pharmaceutical companies may also need a license for other research activities in the drug discovery pipeline.

Does the ‘safe harbor’ research exemption cover the use of CRISPR by pharmaceutical companies?

Under the so-called ‘safe harbor’ provision of the US Hatch-Waxman Act, originally designed to sidestep some of the legal obstacles preventing novel drugs from coming to market, patented technologies can be used without a license if their use is related to the development and submission of information to a regulatory agency.

This has led to the misperception that if a pharmaceutical product that utilizes CRISPR/Cas9 will ultimately be brought to market you don’t need a license. But this is not the case.

For commercial research organizations, the ‘safe harbor’ research exemption only allows for use of patented technology for the express purpose of obtaining data for regulatory purposes related to active pharmaceutical ingredients or medical devices. The exemption does not apply to discovery activities or other research development uses, and legal decisions in this area have become narrower in recent years.

As noted in the landmark 2005 Merck vs Integra ruling, “The Federal Circuit did not provide any bright line rule as to when exactly the safe harbor provision applies. The court observed that the provision does not cover all stages of the drug development simply because some of the drugs will need regulatory approval some day.”

Discovery-phase research activities, such as studying pathways and mechanisms or the creation of animal and /or cell models and vector systems, would not be subject to the ‘safe harbor’ provisions. This means that commercial pharmaceutical companies of any size using CRISPR/Cas9 for research and development purposes will most likely require a license.

If I buy licensed CRISPR reagents, do I need to purchase my own license?

There are many CRISPR/Cas9-based reagents and services on the market.

Generally speaking, use of third-party tools (such as libraries, cells, and animal models created using CRISPR) for internal discovery research, such as screening and pre-clinical studies, does not require a license so long as the provider has a license to convey internal research rights to you.

However, you must check that the vendor you choose is fully licensed. This might include more than one license depending on your location and application. Some providers of CRISPR/Cas9-based products and services are not appropriately licensed, and using their reagents may put your company at risk of future legal action. Buyer beware!

It’s also important to be aware that if you create cell or animal models using CRISPR/Cas9 and then transfer them to a third party, or if you use CRISPR/Cas9 for any commercial purpose such as modifying cells for manufacturing, you need an appropriate license.

We’re an academic research lab using CRISPR/Cas9 – do we need a license?

Research using CRISPR/Cas9 in purely academic contexts has been widely permitted by the patent owners and does not require a license. But any materials created using the technology cannot be used in a commercial environment without a license.

If services or products created using CRISPR/Cas9 are at any point being sold, even to other academic institutions, a license is needed. And commercial organizations receiving CRISPR-modified cells, animals, or other materials from an academic lab must obtain a license to use them.

Keep in mind that University startup companies using CRISPR/Cas9 are not covered by these academic use permissions. ERS Genomics offers affordable licensing for incubators and startups to make sure you’re covered right from the outset.

The bottom line?

CRISPR/Cas9 is a patented technology, so the need to obtain appropriate licenses to use it cannot be ignored. Getting the right license early prevents problems – and potentially costly legal action – further down the line.

Even in the light of the recent patent interference ruling in the US, CVC (University of California, the University of Vienna, and Emmanuelle Charpentier, often referred to as the Berkeley group) still holds the foundational patents covering the use of CRISPR/Cas9 in all cells in the US and many other territories.

The easiest path forward is to obtain a license from ERS Genomics which is authorized to license access to the CVC patents, for any commercial application outside of direct human gene editing therapeutics, including drug discovery and development research.

CRISPR/Cas9 is a revolutionary and exciting technology, and we want to see it being used as widely as possible. Talk to us to get the advice and support you need to make the most of CRISPR to transform your research and develop the life-saving therapies of the future.

As the global licensing leader for CRISPR/Cas9, ERS Genomics is the first port of call when developing a commercial or research application using CRISPR/Cas9. This applies whether you're a biotech start-up or an established life sciences organisation.

[Talk to us today](#) to discuss your licensing needs.