The following Frequently Asked Questions (FAQs) were developed by the Huntington's Disease Society of America (HDSA) to provide general educational information about the recent news about AMT-130. These FAQs are based on information that is currently available and are intended solely for educational purposes. They do not reflect the opinions, views, or positions of uniQure. Individuals are encouraged to speak with their healthcare providers or qualified medical professionals for personalized information and guidance regarding treatment options.

AMT-130: Frequently Asked Questions

What is AMT-130?

AMT-130 is the first gene therapy developed specifically for Huntington's disease (HD). It delivers a piece of genetic material packaged into a harmless virus directly to the brain through surgery. Once inside, these genetic instructions target and destroy the RNA copy of the huntingtin gene. Because the instructions remain in the brain permanently, this is an irreversible procedure.

Why is everyone talking about it?

Recent results from a small study have generated excitement. In 12 people who received a high dose of AMT-130, uniQure reported a **75% slowing of disease progression** after three years. This was measured using the *composite Unified Huntington's Disease Rating Scale (cUHDRS)*, which combines: **function** (daily tasks, work, finances, chores), **movement** (motor symptoms), and **thinking skills** (cognitive tests).

Can I get access to AMT-130?

Not yet. The trial is still ongoing and currently fully enrolled at several U.S. sites. uniQure is collecting long-term data and may expand enrollment later. To stay informed, visit HDTrialfinder.org, where you can search for active HD studies in your area.

Does this mean we've found a cure?

No. The results are encouraging but come from a small group. We don't know if they'll hold up in a larger population. The study compared participants to an **external control group** (people from the Enroll-HD study who did not receive surgery) rather than a placebo. This is necessary because it would not be feasible to perform brain surgery on people who don't receive the treatment.

While this type of comparison is valid, it's not as definitive as a double-blinded trial. Still, several points make the findings promising:

- All measures pointed in the same direction: slower progression.
- A biomarker called **NfL**, which usually increases with brain cell damage, actually **decreased** in these participants.
- Results support the broader idea that **lowering huntingtin** could work, which is good news for other similar therapies in development.

It's important to understand that a disease-modifying therapy is not the same as a cure. A cure would completely stop or reverse the disease. A disease-modifying therapy aims to slow or delay its progression, helping people maintain their abilities and quality of life for longer. AMT-130, if successful, would fall into the second category.

What happened with the FDA and AMT-130?

On November 3, 2025, uniQure announced that the FDA has given new feedback about the path to approval for AMT-130. According to previous uniQure announcements, the FDA had indicated that results from the ongoing Phase I/II study could be compared to data from untreated Enroll-HD participants, and that this comparison might serve as the main evidence supporting a Biologics License Application (BLA). A BLA is the formal request a company submits to the FDA asking for permission to make a biological treatment, such as a gene therapy, available for use in the United States.

However, in a recent meeting, the FDA signaled that this approach may no longer be acceptable. This means the company will need to work with the FDA to understand what additional steps or evidence are required before submitting an application for approval.

The timing of the BLA submission is now uncertain. uniQure has said it was surprised and disappointed by this change but remains committed to working with the FDA to determine a viable path forward.

When could AMT-130 become available?

The original plan was for uniQure to present data and next steps to the FDA in early 2026, which could have led to a potential approval decision later that year. However, following the FDA's recent feedback, that timeline is now uncertain.

uniQure will continue discussions with the FDA to understand what additional information or analyses are needed before it can submit its formal application for approval. The company has stated that it remains committed to finding the fastest path forward to make AMT-130 available to patients, but at this stage, there is **no clear timeline** for when that might happen.

Even if approved, availability will likely be limited to specialized centers, since AMT-130 requires brain surgery.

Should I get genetic testing now?

This is a personal choice. Currently, there are **no approved disease-modifying treatments** for people who test positive. A genetic counsellor can help you think through your options and the emotional, legal, and financial implications.

In the U.S., one major consideration is that a positive test result can affect eligibility for **life, disability, or long-term care insurance**. For more information, visit hdsa.org for genetic testing resources.

How much will it cost?

Gene therapies are extremely expensive—typically **$1–4 million** before insurance.

In the U.S., coverage will depend on private health insurance plans, Medicare, or Medicaid eligibility. Because AMT-130 isn't yet approved or priced, it's too early to know what insurance will cover. uniQure has said they plan to "educate payers on the value of this drug," which may help improve access if the therapy is approved.

Who will be eligible?

So far, the therapy has only been tested in people with **early symptoms** of HD, meaning they have measurable movement, thinking, and function symptoms, but day-to-day function remains largely intact.

If the FDA approves it, eligibility will likely be limited to similar individuals at first. Over time, further studies may explore use in people at later or pre-symptomatic stages. The process takes time, but it's necessary to ensure safety.

Meanwhile, other huntingtin-lowering therapies, including less invasive approaches like pills and spinal-tap delivery, are also in development, so progress is not limited to one avenue.

Why am I feeling so emotional about this news?

You're not alone. Many people feel a mix of hope, grief, and uncertainty. Some are excited about progress, while others feel pain that it came too late for someone they love. These are valid and shared emotions across the HD community.

If you're struggling, please reach out to the **HDSA Helpline (1-800-345-HDSA)** to connect with support groups or social workers. Speaking with a therapist or counsellor can also help you navigate these complex feelings.

What can I do right now?

Joining Enroll-HD is one of the most impactful ways to move research forward. It helps scientists understand how the disease progresses and improves the design of future trials. Participants can also choose to be contacted about new studies.

Action is not limited to research participation. Establish care with an HD-experienced team, ideally at an HDSA Center of Excellence or a clinic with multidisciplinary expertise. Good clinical care improves quality of life today and ensures that, if new treatments become available, you are already connected to providers who can evaluate eligibility.

HDSA has developed relationships with FDA staff and implemented a strategic advocacy program focused on educating the FDA about the needs of the HD community and tolerance for benefit/risk. We are currently working with our D.C. policy firm to evaluate the most appropriate strategy to address this current situation. Stay abreast of HDSA advocacy campaigns at the HDSA Advocacy Hub. Subscribe here to receive timely updates and communications.

The bottom line

The AMT-130 results are a reason for **cautious optimism**. While far from a cure, they suggest that huntingtin-lowering strategies may truly work. Although the regulatory path forward is not totally clear, this is a pivotal moment for HD research—and hopefully just the beginning of more good news to come.

More information

- The First Domino Falls: AMT-130 Gene Therapy Slows Huntington's in Landmark Trial
- Additional Clarity: What We Know 4 Weeks After the uniQure News
- UniQure and FDA No Longer in Alignment on Approval Pathway for AMT-130

If you need support, please don't hesitate to reach out to HDSA's National Helpline at **info@hdsa.org** or **(800) 345-HDSA (4372)**

To reach an HDSA Social Worker: CLICK HERE TO FIND A SOCIAL WORKER IN YOUR AREA

For community support and connection: CLICK HERE TO SEE A LIST OF HDSA ONLINE SUPPORT GROUPS