A CLINICAL TRIAL FOR PATIENTS WITH HEMOPHILIA B
AMT-061 is an investigational gene therapy being developed by uniQure for the treatment of patients with hemophilia B. AMT-061 is currently being studied in the HOPE-B trial for adults with moderately severe or severe hemophilia B.¹
You may have heard about ways scientists have tried to create a gene therapy to treat hemophilia B.

Gene Therapy by uniQure
AMT-061

Read on to learn about AMT-061, an investigational gene therapy that is now in clinical trials.
GENE THERAPY BY UNIQURE:
AMT-061

The AMT-061 gene therapy is composed of two pieces: the capsid and the gene cassette. Together, these form the gene therapy vector.

AAV5 Capsid: The Shipping Box

- The capsid contains certain “shipping” information, so that it can deliver its contents to where they are needed – the liver.
- The capsid is made from a virus – the adeno-associated virus type 5 (AAV5).
- This type of virus does not cause disease.

Factor IX Gene Cassette: The Instruction Manual!

- The gene cassette is found inside the AAV5 capsid. The capsid delivers the gene cassette to the liver cells.
- The cells in the liver will follow the instructions provided by the gene cassette to make Factor IX.
- This instruction manual stays in the liver cells, allowing them to continue making Factor IX for a long time after a single delivery.

GENE THERAPY AT WORK IN HEMOPHILIA B

AMT-061 is an investigational gene therapy that is now being studied in an important clinical trial called HOPE-B. If successful, the data from this clinical study will be used to seek approval of this new gene therapy for hemophilia B patients.¹

This study will test the effects of a single dose of this gene therapy in patients with moderately severe or severe hemophilia B.¹

- HOPE-B will evaluate whether AMT-061 can establish enough production of active Factor IX to prevent bleeding episodes.
- HOPE-B will also look at whether the effect of this gene therapy on bleeding episodes is similar to Factor IX prophylaxis.
- Safety of AMT-061 will be assessed throughout the trial by monitoring adverse events, physical examinations, and laboratory measures.
- HOPE-B will be used to investigate whether a single dose of AMT-061 will provide a long-lasting, durable effect.

ABOUT THE HOPE-B TRIAL

HEALTH OUTCOMES WITH PADUA GENE; EVALUATION IN HEMOPHILIA B

AMT-061 is an investigational gene therapy that is now being studied in an important clinical trial called HOPE-B. If successful, the data from this clinical study will be used to seek approval of this new gene therapy for hemophilia B patients.¹

This study will test the effects of a single dose of this gene therapy in patients with moderately severe or severe hemophilia B.¹
WHO IS ELIGIBLE TO PARTICIPATE IN HOPE-B?

Patients may be able to participate in the study if they are:

- Male and at least 18 years old
- Diagnosed with moderately severe or severe congenital hemophilia B (≤2% Factor IX activity)
- Patients with >150 previous exposure days of treatment with Factor IX protein

Patients will not be able to participate in this study if they have:

- Developed inhibitors to Factor IX at any time
- Certain abnormal laboratory test results
- A positive test for human immunodeficiency virus, not controlled with antiviral therapy
- An active infection with hepatitis B or C virus
- History of hepatitis B or C exposure and currently receiving antiviral therapy at the last visit prior to dosing

For more information, please visit www.Clinicaltrials.gov or contact uniQure at uniQureHOPE-B@uniqure.com.

WHY SHOULD PATIENTS CONSIDER ENROLLING IN THIS TRIAL?

Clinical trials are essential to the advance of medicine.

- Clinical trials are used to find out whether new treatments and prevention measures are safe and effective for patients.
- The results of clinical trials are used by regulatory agencies to determine whether a new medical therapy can be approved.
- Without patients, clinical trials cannot happen.
- Study participants actively contribute to the development of new therapies for other patients in the future.

Participation in HOPE-B may help bring a novel therapy to the hemophilia B community in the future.

WHAT WILL HAPPEN DURING THE TRIAL?

There are 5 phases of the HOPE-B trial

Screening
1 visit

- Confirm patient eligibility

Lead-In Phase
≥6 months

- Monitor health of study participants
- Collect information on disease management

Dosing
1 visit

- Administer a single infusion of AMT-061 to study participants
- Monitor vital signs
- Collect blood samples

Post-Treatment Follow-Up
1 year

- Regular follow-up visits for patient safety and efficacy

Long-Term Follow-Up
4 years

- Infrequent follow-up visits for patient safety and efficacy
For more information, please visit www.Clinicaltrials.gov or contact uniQure at uniQureHOPE-B@uniqure.com.

References