



uniQure Announces Clinical Data Presentations at the 62nd American Society of Hematology (ASH) Annual Meeting

November 4, 2020

LEXINGTON, Mass. and AMSTERDAM, Nov. 04, 2020 (GLOBE NEWSWIRE) -- [uniQure N.V.](https://www.unicure.com) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that four data presentations, including one oral presentation and three poster presentations, will be delivered at the 62nd American Society of Hematology (ASH) Annual Meeting, which will be held December 5-8 as a virtual event.

"We expect to have a strong presence at the virtual ASH annual meeting next month, and look forward to having long-term follow-up data presented from our hemophilia B gene therapy studies, including two years of follow-up on the Phase IIb clinical trial of etranacogene dezaparvovec (AMT-061) and up to five years of follow-up from the Phase I/II clinical trial of AMT-060," stated [Matt Kapusta](#), chief executive officer at uniQure. "We also remain on track to announce top-line data from the pivotal HOPE-B study of etranacogene dezaparvovec before the end of this year, either at ASH or through a company presentation."

Oral Presentation

Title: *Etranacogene Dezaparvovec (AAV5-Padua hFIX variant), an Enhanced Vector for Gene Transfer in Adults with Severe or Moderate-Severe Hemophilia B: Two Year Data from a Phase 2b Trial*

Presenter: Annette von Drygalski, M.D., PharmD

Session Name: 801. Gene Editing, Therapy and Transfer I

Date: Monday, December 7, 2020

Presentation Time: 2:45 p.m. ET (11:45 a.m. PT)

Poster Presentations

Title: *AMT-060 Gene Therapy in Adults with Severe or Moderate-Severe Hemophilia B Confirm Stable FIX Expression and Durable Reductions in Bleeding and Factor IX Consumption for up to 5 Years*

Session Name: 801. Gene Editing, Therapy and Transfer: Poster III

Date: Monday, December 7, 2020

Presentation Time: 10:00 a.m. - 6:30 p.m. ET (7:00 a.m. - 3:30 p.m. PT)

Title: *A Single Administration of AAV5-hFIX in Newborn, Juvenile and Adult Mice Leads to Stable hFIX Expression up to 18 Months after Dosing*

Session Name: 801. Gene Editing, Therapy and Transfer: Poster II

Date: Sunday, December 6, 2020

Presentation Time: 10:00 a.m. - 6:30 p.m. ET (7:00 a.m. - 3:30 p.m. PT)

Title: *Examining the Hemophilia Disability Paradox*

Session Name: 904. Outcomes Research—Non-Malignant Conditions: Poster II

Date: Sunday, December 6, 2020

Presentation Time: 10:00 a.m. - 6:30 p.m. ET (7:00 a.m. - 3:30 p.m. PT)

The conference abstracts were made available today and can be accessed through this link: [ASH abstracts](#).

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. We are leveraging our modular and validated technology platform to rapidly advance a [pipeline](#) of proprietary gene therapies to treat patients with hemophilia B, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other diseases. www.unicure.com

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, whether we will present long-term follow-up data from our hemophilia B gene therapy studies, including two years of follow-up on the Phase IIb clinical trial of etranacogene dezaparvovec (AMT-061) and up to five years of follow-up from the Phase I/II clinical trial of AMT-060, and whether we will announce top-line data from the pivotal HOPE-B study of etranacogene dezaparvovec before the end of this year. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our and our collaborators' clinical development activities, clinical results, collaboration arrangements, corporate reorganizations and strategic shifts, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's Quarterly Report on Form 10-Q filed on October 27, 2020. Given these risks, uncertainties and

other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

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