KURZPROTOKOLL
SHP655-201

Öffentlicher Titel	Phase II Studie zu rADAMTS-13 bei thrombotischer thrombozytopenischer Purpura (TTP)
Wissenschaftl. Titel	A Phase 2, multicenter, randomized, placebo-controlled, doubleblind study in patients with acquired thrombotic thrombocytopenic purpura (aTTP) to evaluate the pharmacokinetics, safety, and efficacy of rADAMTS-13 (SHP655) administered in addition to standard of care (SoC) treatment
Kurztitel	SHP655-201
Studienart	multizentrisch, prospektiv, Therapiestudie, randomisiert, Pharma-Studie, doppelblind, zweiarmig
Studienphase	Phase II
Erkrankung	Blut: Blutbildungs- und Blutabbaustörungen
Einschlusskriterien	- Participant or legally authorized representative voluntarily signs informed consent. For participants unable to provide consent, a fully recognized medical proxy may be used according to local laws. - Participant is 18 to 75 years old at the time of screening. - Participant has been diagnosed with primary or secondary autoimmune acquired thrombotic thrombocytopenic purpura (aTTP) based on the following criteria: a) Thrombocytopenia [drop in platelet count greater than or equal to (>=) 50% or platelet count lesser than (<) 100,000/microlitre (L)] i) No more than 3 participants per arm may be enrolled with a screening platelet count >= 50,000/L. b) Microangiopathic hemolytic anemia [elevation of lactate dehydrogenase (LDH) greater than (>) 2-fold or by presence or increase of schistocytes in peripheral blood smear]. - Willingness to fully comply with study procedures and requirements, and intention to initiate plasma exchange (PEX). Participants may be provisionally entered into the trial and undergo randomization pending the results of the ADAMTS-13 activity, anti-ADAMTS-13 antibody, and genetic testing for congenital thrombotic thrombocytopenic purpura (cTTP). - If female of childbearing potential, participant presents with a negative pregnancy test and agrees to employ adequate birth control measures for the duration of the study. Sexually active males must use an accepted and effective method of contraception during the treatment and until a minimum of 16 days after the last dose administered.
Ausschlusskriterien	- Participant has been diagnosed with congenital TTP. - Participant has plasma ADAMTS-13 activity > 10% of normal at the central lab; if screening samples are not taken until after the first PEX, ADAMTS-13 activity from the local lab is permitted to determine eligibility. - Participant has been diagnosed with another cause of thrombotic microangiopathy (TMA) including: DIC, disseminated malignancy, malignant hypertension, hematopoietic stem cell transplantation, shiga toxin related and atypical HUS, drug toxicity (e.g. gemcitabine, mitomycin C, clopidogrel) and pregnancy-related thrombocytopenia syndromes (e.g. HELLP, eclampsia). - Participant has been exposed to another IP within 30 days prior to enrollment or is scheduled to participate in another clinical study involving IP or investigational device during the course of the study. - Participant has received caplacizumab within 1 month prior to study enrollment. - Participant is human immunodeficiency virus positive (HIV+) with unstable disease or CD4+ count lesser than or equal to (<=) 200 cells/mm3 within 3 months screening. - Participants with conditions of severe immunodeficiency. - Participant has had a previous aTTP event in the past 30 days. - Participant has another underlying progressive fatal disease and/or life expectancy of less than 3 months.

	- Participant is identified by the investigator as being unable or unwilling to cooperate with study procedures - Participant suffers from a mental condition rendering him/her unable to understand the nature, scope, and possible consequences of the study and/or evidence of an uncooperative attitude. However, a fully recognized medical proxy will be permitted to provide consent. - If female, participant is pregnant or lactating. - Participant is a family member or employee of the Sponsor or investigator. - Any contraindication to PEX, methylprednisolone and/or rituximab as per prescribing information. - Known life-threatening hypersensitivity reaction, including anaphylaxis, to the parent molecule ADAMTS-13, hamster protein, or other constituents of SHP655.
Alter	18 - 75 Jahre
Sponsor	Shire Pharmaceutical Development Ltd.
Registrierung in anderen Studienregistern	EudraCT 2018-003775-35 ClinicalTrials.gov NCT03922308 (primäres Register)