KURZPROTOKOLL
CMBG453B12301

Öffentlicher Titel	Phase III Studie zu Azacitidin und MBG453 bei myelodysplastischen Syndromen (MDS) oder chronischer myelomonozytärer Leukämie (CMML)
Wissenschaftl. Titel	A randomized, double-blind, placebo-controlled phase III multi-center study of azacitidine with or without MBG453 for the treatment of patients with intermediate, high or very high risk myelodysplastic syndrome (MDS) as per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Kurztitel	CMBG453B12301
Studienart	multizentrisch, prospektiv, Therapiestudie, randomisiert, doppelblind, zweiarmig
Studienphase	Phase III
Erkrankung	Blut: Myeloische Neoplasien/Dysplasien: Chronische myelomonozytäre Leukämie (CMML) Blut: Myeloische Neoplasien/Dysplasien: Myelodysplastische Syndrome (MDS)
Einschlusskriterien	- Signed informed consent must be obtained prior to participation in the study - Age >= 18 years at the date of signing the informed consent form - Morphologically confirmed diagnosis of myelodysplastic syndrome (MDS) based on WHO 2016 classification (Arber et al 2016) by local investigator assessment with one of the following Prognostic Risk Categories, based on the revised International Prognostic Scoring System (IPSS-R): o Very high (> 6 points) o High (> 4.5 - <= 6 points) o Intermediate (> 3 - <= 4.5 points) Or Morphologically confirmed diagnosis of Chronic Myelomonocytic Leukemia -2 based on WHO 2016 classification (Arber et al 2016) by local investigator assessment with WBC < 13 x 109/L - Indication for azacitidine treatment according to the investigator, based on local standard medical practice and institutional guidelines for treatment decisions - Not eligible at time of screening for intensive chemotherapy according to the investigator, based on local standard medical practice and institutional guidelines for treatment decisions, including assessment of individual clinical factors such as age, comorbidities and performance status - Not eligible at time of screening for hematopoietic stem cell transplantation (HSCT) according to the investigator, based on local standard medical practice and institutional guidelines for treatment decisions, including assessment of individual clinical factors such as age, comorbidities, performance status, and donor availability - Eastern Cooperative Oncology Group (ECOG) performance status of 0, 1 or 2
Ausschlusskriterien	- Prior exposure to TIM-3 directed therapy at any time. Prior therapy with immune checkpoint inhibitors (e.g, anti-CTLA4, anti-PD-1, anti-PD-L1, or anti-PD-L2), cancer vaccines is allowed except if the drug was administered within 4 months prior to randomization - Previous first-line treatment for intermediate, high, very high risk myelodysplastic syndromes (based on IPSS-R) or CMML with any antineoplastic agents including for example chemotherapy, lenalidomide and hypomethylating agents (HMAs) such as decitibine and azacitidine. However, previous treatment with hydroxyurea or leukopheresis to reduce WBC count is allowed prior to randomization - Investigational treatment received within 4 weeks or 5 half-lives of this investigational treatment, whatever is longer, prior to randomization. In case of a checkpoint inhibitor: a minimal interval of 4 months prior to randomization is necessary to allow randomization - Subjects with Myelodysplastic syndrome (MDS) based on 2016 WHO classification (Arber et al 2016) with revised International Prognostic Scoring System (IPSS-R) <= 3 - Diagnosis of acute myeloid leukemia (AML) including acute promyelocytic leukemia and extra-medullary acute myeloid leukemia, primary or secondary myelofibrosis based on WHO 2016 classification (Arber et al 2016)

	- Diagnosis of therapy related myeloid neoplasms based on WHO 2016 classification (Arber et al 2016) - History of organ or allogeneic hematopoietic stem cell transplant
Alter	18 Jahre und älter
Prüfzentren	**Universitätsklinikum Frankfurt** (Rekrutierung beendet) Medizinische Klinik II, Hämatologie/Onkologie Theodor-Stern-Kai 7 60590 Frankfurt am Main Dr. med. Jörg Chromik Tel: 069 6301-5677 Fax: 069 6301-6089 joerg.chromik@kgu.de
Sponsor	Novartis Pharma
Registrierung in anderen Studienregistern	ClinicalTrials.gov NCT04266301 (primäres Register) EudraCT 2019-002089-11
Links	Studiendokumente zum Download (roXtra)