KURZPROTOKOLL
MPN-SG 01-09

Öffentlicher Titel	Phase II Studie zu Pomalidomide bei MPN mit Fibrose
Wissenschaftl. Titel	Multi-Center Phase II Study with Pomalidomide in Patients with Myeloproliferative Neoplasms in Fibrotic Stage
Kurztitel	MPN-SG 01-09
Studienart	multizentrisch, prospektiv, offen/unverblindet, einarmig, Investigator Initiated Trial (IIT)
Studienphase	Phase II
Erkrankung	Blut: Myeloische Neoplasien/Dysplasien: Myeloproliferative Neoplasien (MPN)
Ziele	- To evaluate clinical efficacy (disease response) of pomalidomide in MF patients based on the consensus criteria of the International Working Group for Myelofibrosis Research and Treatment (IWG-MRT) - To evaluate the safety profile of pomalidomide in MF patients - To assess predictive response parameters using cytogenetic and molecular genetic analyses including microarray-based technipues - To assess overall clinical outcome
Einschlusskriterien	- Age > 50 years at the time of voluntarily signing an IRB/IEC-approved informed consent - Diagnosis of Myeloproliferative Neoplasms (MPN) either de novo myelofibrosis according to WHO criteria (PMF) [20], secondary myelofibrosis (post-PV MF and post -ET MF according to the IWG-MRT consensus terminology) [21] or unclassifiable MPN with biopsy proven myelofibrosis - Anemia with haemoglobin level of < 10 g/dl or transfusion-dependent anemia and/or thrombocytopenia <50 /nl or transfusion-dependent thrombocytopenia and/or neutropenia < 1.0 /nl - Splenomegaly (> 11 cm diameter) and/or leukoerythroblastosis - Adequate organ function, i.e. ALT and/or AST < 3x upper limit of normal (ULN), total bilirubin < 3x ULN and serum creatinine <2 mg/dl - Subject must be willing to receive transfusion of blood products - ECOG performance status <3 - Female subjects with non-childbearing potential°, - must have one medically supervised negative serum or urine pregnancy tests prior to starting study drug - Male subjects – must agree to use condoms throughout study drug therapy, during any dose interruption and for four weeks after cessation of study therapy if their partner is of childbearing potential and has no contraception – even if they have undergone a successful vasectomy. - must agree not to donate semen during study drug therapy and for 90 days following discontinuation of pomalidomide. - Alls subjects (males and females) – will be counselled about potential teratogenic risks of the study medication. . must agree to abstain from donating blood while taking study drug therapy and for at least 28 days following discontinuation of pomalidomide.- must agree not to share study medication with another person and to return all unused study drug to the investigator – only enough pomalidomide for one cycle of therapy may be dispensed with each cycle of therapy.
Ausschlusskriterien	- 1. Females of childbearing potentials°, pregnant or breast feeding females - 2. BCR/ABL – positivity - 3. Diagnosis of ET (according to WHO 2008 criteria) - 4. Diagnosis of PV (according to WHO 2008 criteria) - 5. > 20% blasts in peripheral blood or bone marrow - 6. Known positive status for HIV, HBV or HCV

	- 7. Prior treatment with IMiDs (thalidomide, lenalidomide) or with Interferon-alpha within a 3 month time period before screening - 8. History of thrombosis or pulmonary embolism - 9. Peripheral neuropathy > grade 1 CTC - 10. No consent for registration, storage and processing of the individual disease-characteristics and course as well as information of the family physician about study participation. - 11. Presence of any medical/psychiatric condition or laboratory abnormalities which may limit full compliance with the study, increase the risk associated with study participation or study drug administration, or may interfere with the interpretation of study results and, in the judgement of the Investigator, would make the patient inappropriate for entry into this study - 12. Drug or alcohol abuse within the last 6 months - 13. Criteria for women of non-childbearing potential: A female patient or a female partner of a male patient is considered to have childbearing potential unless she meets at least one of the following criteria: - - Age > 50 years and naturally amenrrhoeic for at least 24 consecutive months (amenorrhoea following cancer therapy does not rule out childbearing potential) - - Premature ovarian failure confirmed by a specialist gynaecologist - - Previous bilateral salpingo-oophorectomy, or hysterectomy - - XY genotype, Turner syndrome, uterine agenesis
Alter	50 Jahre und älter
Prüfzentren	**Universitätsklinikum Frankfurt** (Rekrutierung beendet) Medizinische Klinik II, Hämatologie/Onkologie Theodor-Stern-Kai 7 60590 Frankfurt am Main Anja Binckebanck Tel: 069 6301-6221 Fax: 069 6301-7463 binckebanck@em.uni-frankfurt.de
Förderer	Celgene GmbH
Registrierung in anderen Studienregistern	EudraCT 2009-010738-23
Therapie	Monotherapie mit Pomalidomide