KURZPROTOKOLL
Treo-nicht-maligne

Öffentlicher Titel	Phase II Studie zur Treosulfan-Konditionierung vor Stammzelltransplantation bei nicht-bösartigen Erkrankungen
Wissenschaftl. Titel	Clinical phase II trial to compare Treosulfan-based conditioning therapy with Busulfan-based conditioning prior to allogeneic haematopoietic stem cell transplantation (HSCT) in paediatric patients with non-malignant diseases
Kurztitel	Treo-nicht-maligne
Studienart	prospektiv, Therapiestudie, randomisiert, offen/unverblindet, Pharma-Studie, zweiarmig
Studienphase	Phase II
Erkrankung	Kinder: sonstige
Einschlusskriterien	- Non-malignant disease indicated for first myeloablative allogeneic HSCT, including inborn errors of metabolism, primary immunodeficiencies, haemoglobinopathies and bone marrow failure syndromes - First allogeneic HSCT - Available matched sibling donor (MSD), matched family donor (MFD) or matched unrelated donor (MUD). For bone marrow (BM) and peripheral blood (PB) match is defined as at least 9/10 allele matches after four digit typing in human leucocyte antigen (HLA)-A, -B, -C, -DRB1 and DQB1 antigens. For umbilical cord blood (UCB) match is defined as at least 5/6 matches after two digit typing in HLA-A and -B and four digit typing in DRB1 antigens
Ausschlusskriterien	- Second or later HSCT - HSCT from mismatched donor (less than 9/10 BM/peripheral blood stem cells (PBSC) or less than 5/6 matched cord donor) - Preterm newborn infants (<37 weeks gestational age) and term newborn infants aged 0 - 27 days at time of registration - Obese paediatric patients with body mass index weight (kg)/[height (m)]^2 > 30 kg/m^2 - Diagnosis of Fanconi anaemia and other chromosomal breakage disorders, radiosensitivity disorders (deoxyribonucleic acid (DNA) Ligase 4, Cernunnos- X-ray repair cross-complementing protein 4 (XRCC4) like factor (XLF), Nijmegen Breakage Syndrome (NBS)) and Dyskeratosis Congenita
Alter	< 18 Jahre
Sponsor	Medac GmbH
Registrierung in anderen Studienregistern	ClinicalTrials.gov NCT02349906 (primäres Register) EudraCT 2013-005508-33