# *KURZPROTOKOLL*
# *BALDER Trial*

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| **Öffentlicher Titel** | Phase II Studie zu Erstlinienbehandlung von akuter Graft-versus-Host-Erkrankung mit mesenchymalen Stromazellen MC0518 |
| **Wissenschaftl. Titel** | A randomised, open-label controlled, multicentre, phase 2 trial of first-line treatment with mesenchymal stromal cells MC0518 versus best available therapy in paediatric participants with steroid-refractory acute graft-versus-host disease after allogeneic stem cell transplantation |
| **Kurztitel** | BALDER Trial |
| **Studienart** | multizentrisch, prospektiv, randomisiert, offen/unverblindet, Pharma-Studie, zweiarmig, kontrolliert |
| **Studienphase** | Phase II |
| **Erkrankung** | Kinder: Leukämien und Lymphome: Stammzelltransplantation |
| **Einschlusskriterien** | - Participant had a previous allogeneic HSCT as indicated for non-malignant (including inborn errors of metabolism, primary immunodeficiencies, haemoglobinopathies, and bone marrow failure syndromes) or hematological malignant disease or neuroblastoma<br>- Participant has been clinically diagnosed with Grade II to IV aGvHD according to Harris et al. A biopsy of the involved organs with aGvHD is encouraged but not required<br>- Participant has experienced failure of previous first-line aGvHD treatment (that is, SR -aGvHD), defined as: 1. aGvHD progression within 3 to 5 days of therapy onset with >=2 milligram per kilogram per day (mg/kg/day) of prednisone equivalent or; 2. failure to improve within 5 to 7 days of treatment initiation with >=2 mg/kg/day of prednisone equivalent or; 3. incomplete response after greater than (>) 28 days of immunosuppressive treatment including at least 5 days with >=2 mg/kg/day of prednisone equivalent<br>- Male or female participant who is >=28 days and <18 years of age and has a minimum body weight of 3.2 kilograms (kg) at the Screening Visit<br>- Participant has an estimated life expectancy of >28 days<br>- Participant, if female and of childbearing potential, agrees to use a highly effective contraceptive measure starting at the Screening Visit and continuing throughout the entire trial period<br>- Participant, if a fertile male, agrees to sexual abstinence or to use a condom during sexual activity with their female partner of childbearing potential or pregnant partner. Additionally, if their partner is a woman of childbearing potential (WOCBP), then their partner must use an additional highly effective contraceptive method during sexual activity starting at the Screening Visit and continuing throughout the entire trial period<br>- A written informed consent of the participant's parent(s) / legal guardian(s) (and participant's assent, when applicable) has been obtained according to national regulations |
| **Ausschlusskriterien** | - Participant has overt relapse or progression or persistence of the underlying disease<br>- Participant has received the last HSCT for a solid tumor disease other than neuroblastoma<br>- Participant has graft-versus-host disease overlap syndrome<br>- Participant has received systemic first-line treatment for aGvHD other than steroids and a prophylaxis with other than calcineurin inhibitors, mammalian target of rapamycin (mTOR) inhibitors, anti-thymocyte globulin, mycophenolate mofetil, methotrexate, abatacept, or cyclophosphamide. Note: In vitro or in vivo graft manipulation to prevent graft-versus-host disease (example, T-cell depletion) during HSCT is permitted. Restart of initial prophylaxis with calcineurin inhibitors, mammalian target of rapamycin inhibitors, or mycophenolate mofetil after aGvHD onset is permitted |

- Participant has received prior mesenchymal stromal cell (MSC) treatment, including MC0518/Obnitix®
- Participant has a known pregnancy (as confirmed by a positive pregnancy test result at the Screening Visit) and / or is breastfeeding
- Participant has a known hypersensitivity to MC0518 and / or its excipients (dimethyl sulfoxide, human serum albumin, isotonic sodium chloride solution)
- Participant has a known hypersensitivity or any contraindication to the Investigator's choice BAT (extracorporeal photopheresis, anti thymocyte globulin, etanercept, infliximab, or ruxolitinib) and / or its excipients. For a list of excipients please refer to the respective Summary of Product Characteristics
- Participant has an underlying or current medical or psychiatric condition that, in the opinion of the Investigator, would interfere with the evaluation of the participant
- Participant has an uncontrolled infection (examples, sepsis or multi-organ failure) including significant bacterial, fungal, viral, or parasitic infection requiring treatment
- Participant has received treatment with any other investigational agent within 30 days or 5 half-lives (whichever is longer) before the Screening Visit

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| **Alter** | 1 Monat bis 17 Jahre |
| **Prüfzentren** | **Universitätsmedizin Frankfurt** (Aktiv)<br>Klinik für Kinder- und Jugendmedizin<br>Theodor-Stern-Kai 7<br>60590 Frankfurt am Main<br>Prof. Dr. med. Peter Bader<br>Tel: 069 6301-7542<br>Fax: 069 6301-4202<br>peter.bader@unimedizin-ffm.de |
| **Sponsor** | Medac GmbH |
| **Registrierung in anderen Studienregistern** | ClinicalTrials.gov NCT06075706<br>EudraCT 2023-503952-28-00 |
| **Links** | Zu den Ein- und Ausschlusskriterien |