New data confirming Myelo001 efficacy in Acute Radiation Syndrome (ARS) presented at 65th annual meeting of the Radiation Research Society.


The primary manifestation of ARS is the depletion of hematopoietic stem cells, constituting one of the major causes of mortality. Three radiomitigators - G-CSF, Peg-G-CSF and GM-CSF- were approved by FDA as medical countermeasures (MCM) for hematopoietic-ARS (H-ARS) but are confined to therapeutic injections after irradiation >2 Gy and their effects are primarily limited to neutrophils and macrophages. Therefore, an unmet need prevails to protect lymphocytes and thrombocytes for functional hemostasis. Moreover, instability at room temperature and the administration route limit their wider use in a mass casualty scenario. Here we present the data on the efficacy of a novel drug candidate under development for reduction of mortality and myelosuppression after exposure to ionizing radiation.

Imidazolyl ethanamide pentandioic acid (IEPA, Myelo001) is a small molecule drug delivered as an oral formulation that is stable at room temperature for at least 3 years. Preclinical and clinical studies showed that IEPA has both prophylactic and therapeutic efficacy at reducing hematopoietic symptoms caused by radiation and chemotherapy. In 5 in vivo studies in irradiated mice and rabbits, IEPA reduced the nadir and accelerated recovery of neutrophils, lymphocytes, thrombocytes, and erythrocytes. In mice, treatment 24h post-total-body-irradiation resulted in increased survival (86% IEPA vs. 56% control), faster bone marrow recovery and reduced body weight loss. Moreover, IEPA treatment prior to radiochemotherapy or chemotherapy led to a faster recovery of white blood cells in human subjects.

Comprehensive chronic toxicology and safety studies, as well as clinical studies in over 3,000 patients in other indications, displayed a positive safety and tolerability profile for orally applied IEPA. Thus, combined characteristics of IEPA make it a promising MCM candidate for radioprotection and radiomitigation that is easy to distribute and use. The acceleration of the lymphocyte recovery suggests the use as a potential therapy in the prevention and treatment of Delayed Effects of Acute Radiation Exposure (DEARE). Future research includes translational radiation studies in the NHP model and investigation of higher doses, altered posologies and polypharmacy approaches to optimize efficacy.

About Myelo Therapeutics GmbH:
Myelo Therapeutics is a pharmaceutical company based in Berlin and Dresden, Germany, that is developing innovative treatments in areas of high unmet medical needs. For more information, visit www.myelotherapeutics.com.

Contact: Myelo Therapeutics GmbH; Till Erdmann, Rheinsberger Straße 7; 10115 Berlin, Germany
Phone: +49 (0) 170 9126 402; info@myelotherapeutics.com