

## Intravenous Busulfan Offers Hope To Leukemia Patients

University of Texas



Back in 1990, Borje S. Andersson, M.D, Ph.D., recognized that lethal liver failure in one of every four to five patients undergoing stem cell transplantation for leukemia was unacceptable.

He traced this to the unpredictable effect of high-dose busulfan given by mouth while preparing the patients for their transplant. He came up with the idea of giving this “insoluble drug” directly into the blood, something thought to be impossible, since busulfan is chemically unstable and thought of as truly insoluble. He suggested that an intravenous busulfan formulation would be easier and safer to administer. Not accepting that busulfan would be insoluble, Dr. Andersson set out to create an intravenous formulation.

This project led to the filing of a patent application for “intravenous busulfan” three years later, and in 1995 clinical studies were initiated to better prepare leukemia patients for stem cell transplants. The clinical trials spearheaded by The University of Texas M. D. Anderson Cancer Center were carried out at seven different U.S. transplant centers, and in 1999 the U.S. Food and Drug Administration (FDA) granted regulatory approval for intravenous busulfan (IV Busulfex).

“ *This was the first, and so far the only, time that the FDA approved a chemotherapeutic agent or technology for use in pretransplantation preparative therapy.*

Since then, the transplant group at M. D. Anderson has conducted a series of clinical trials using IV Busulfex, combined with either cyclophosphamide or with Fludarabine as part of conditioning therapy for patients with chronic and acute myeloid leukemia and for patients with myelodysplastic syndrome. They have firmly established IV Busulfex™ as a safer alternative to existing pretransplant preparative regimens for patients with myeloid leukemia; the risk of a lethal complication in an adult undergoing a transplant for leukemia is now below eight percent in the first year after the transplant. IV Busulfex is now successfully used in more than 40 countries around the globe.

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