



Biology of Blood and Marrow Transplantation



journal homepage: www.bbmt.org

ASTCT Notes

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FACT's Chief Medical Officer Reflects on Standards, Impacting Patients, and What's Next for Cellular Therapies

More than 25 years ago, a group of physicians, scientists, clinicians, and researchers came together. They had one mission in mind: As blood and marrow transplantation was growing and evolving as a field in the early 1990s, there were growing concerns about how it would be regulated. The FDA had announced its regulatory approach to bone marrow transplantation, and leaders in the field wanted to be ahead of the curve.

"There was a strong belief that the professionals in the field should be setting the standards, not the regulators," said Dr. Phyllis Warkentin, FACT's chief medical officer and one of the founding members. The newly-formed ASBMT (now known as ASTCT) and ISHAGE (now known as the International Society for Cell and Gene Therapy, or ISCT) each had its own guidelines for clinical and laboratory practice. They decided to combine their efforts.

"They realized the value of a comprehensive set of guidelines and standards that would address the full spectrum of the therapy, with patient outcome as the primary quality end point," she said.

And thus was born the Foundation for Accreditation of Cellular Therapy, or FACT, the standards-setting and accreditation arm of ASTCT and ISCT. FACT establishes standards for high quality medical and laboratory practice in cellular therapies. Over 90% of all eligible U.S. facilities and programs are FACT accredited, and the standards are used internationally.

Warkentin has been the FACT medical director there since its inception. She said those during early days, there was a lot of collaboration, some disagreement, but much compromise and consensus building. There was an overall sense that to establish high quality standards for

BMT and cellular therapy centers was a critical mission. It was a barebones operation when it started, but one everyone knew was important.

"There was no office, there were no staff, no one was paid," she said. "The entire effort was on our volunteer time, particularly that of the founding Board of Directors."

The first set of standards was drafted in 1996. Warkentin said it was evidence-based wherever possible, and focused on processes and outcomes rather than being too prescriptive. Standards were flexible, allowing the possibility of many ways to be compliant. They were comprehensive in breadth and depth, including the entire cell therapy from donor selection to product collection, processing, storage, release, administration to the patient, and to patient outcome. She said the founders were intent on promoting quality standards while continuing to foster research and development in the field.

"We weren't trying to discriminate among transplant programs, and we weren't trying to impede new ones starting," she said. "We were encouraging everyone to get to a certain level of quality."

Every three years the standards are reviewed and released as a draft, allowing widespread input from the public, professionals in the field, payers, industry and regulators. Warkentin said they also consider where the field is moving, and what new things need to be addressed in future editions.

This collegial attitude has led FACT to several great partnerships. The first was with JACIE, the Joint Accreditation Committee of ISCT and EBMT. Since the third edition of hematopoietic cellular therapy standards, these Standards have been jointly developed by FACT and JACIE. JACIE independently administers the accreditation program in Europe.

Another important collaboration is with the International NetCord Foundation, now aligned under the World Marrow Donor Association. NetCord was originally established by leaders in cord blood banking to globally unite quality cord blood units and potential recipients.

"This was important because, at that time, there was no single point of access for cord blood units such as now exists in the National Marrow Donor Program. If you wanted cord blood for your patient, you had to contact each bank individually," she said.

In 2000, FACT and the International NetCord Foundation published the first edition of the NetCord-FACT International Standards for Cord Blood Collection, Banking and Release for Administration. FACT subsequently established the voluntary accreditation program for cord blood banks worldwide. Warkentin said she's amazed at how the field and the quality of care have changed just in her lifetime.

She recently had the opportunity to meet a young lady who received an unrelated donor marrow transplant in Australia 20 years ago. The donor was found, and the marrow collected, halfway around the world in Omaha, Nebraska. The recipient visited Omaha this winter for her donor's 70th birthday, and she shared her story to open FACT's Inspector Training Workshop in Melbourne, Australia in May.

As this patient told her story, Warkentin realized that some of the negative experiences from that transplant are issues now addressed, and hopefully therefore improved, by the FACT-JACIE Standards. Other issues highlight the opportunities for continued Standards development. "It was so inspirational and so personal and so detailed – like it happened yesterday," Warkentin said of the woman's story. "It was quite

amazing. It focused us on why we do this work—to continue to improve the quality of medical care and laboratory services for these patients.

Warkentin said moving forward, the advances in the field—specifically those related to new and innovative cell therapy products such as CAR-T cells—are going to shape the future conversation surrounding standards. It will require even more collaboration, with other partners in industry, academia, and those in other disciplines who are using cell therapy. It is interesting to note it is another period of evolving regulatory pathways, and one where Standards can make a difference.

Being part of FACT has been a wonderful experience for Warkentin, one she is proud of on both a personal and professional level.

“I love this organization and its mission,” she said. “It’s cliché to say it’s a passion, but I really love that it makes a difference. The feedback we always get is that people think their programs are better because of FACT accreditation.”

“I am fortunate to have had this work entrusted to me. But the success of FACT is really due to its many volunteers who perform inspections, serve on committees, develop standards, and contribute their expertise to FACT’s educational initiatives—and to the programs that choose to pursue accreditation and qual-

ity medical and laboratory care for their patients.”

WHAT’S NEW AT ASTCT

Join Us for the 6th Annual Clinical Education Conference for NPs, PAs and Fellows

The American Society for Transplantation and Cellular Therapy (ASTCT) in collaboration with Washington University School of Medicine in St. Louis and National Marrow Donor Program / Be The Match® is proud to announce the 6th Annual ASTCT Fall Clinical Education Conference. The conference will be held September 19-21, 2019 at the Washington University School of Medicine in St. Louis, MO. This multi-day conference for NPs, PAs, nurses, fellows and junior faculty will be focused on the care of blood and marrow transplant and cell therapy patients.

For more information, visit astct.org.

ASTCT Pharmacy SIG Publishes Additions to eBook

The Beyond Fundamentals of HCT eBook is now available to be purchased. This eBook is comprised of 31 chapters detailing pharmacotherapy in the hematopoietic cell transplantation (HCT) setting. Recent additions to the eBook include CAR-T Therapy. This is an excellent opportunity to learn more about skills required to care for patients under-

going HCT, with a focus on pharmacotherapeutic management throughout the transplant process. You can purchase the eBook for \$245.00.

To learn more, visit <http://bit.ly/PharmacySIGeBook>.

The New Investigator Award Deadline Moved to September

The ASTCT New Investigator Award in designed to encourage clinical or laboratory research by young investigators in the BMT field. The award is \$30,000 per year, typically for two years, and is preferably to be used to support the investigator’s salary for his or her research effort. Alternatively, the award may be used for direct support of research costs. Submissions on the following topics are preferred:

- Clinical stem cell transplantation
- Cellular therapies beyond transplant
- Conditioning regimens
- Infectious prophylaxis and/or infectious complications of transplant
- Preclinical models of transplant
- Stem cell biology
- Transplantation immunology, including but not limited to GvHD and GvT/GvM
- Survivorship and clinical outcomes

To learn more, visit astct.org.