(reflective music) >> So as the term implies, blood and marrow transplantation is the transplantation or infusion of cells collected either from the blood or from the bone marrow.

The cells in question are blood stem cells, cells that are going to go on and be able to create all of the cells in the blood and immune system lineage.

And that's an important feature to delineate, that they're the stem cells that we're actually looking for.

Stem cells can be collected from the bone marrow, which can be referred to as the stem cell factory, if you will, but they can also be collected from the peripheral blood, and they can also be collected from the placenta, or the cord blood.

So the combination of any one of those sources is an opportunity to transplant, transfuse, these blood stem cells into a patient who may benefit from such a procedure.

(reflective music) So blood stem cells can either be collected from the patient who ultimately receives them, or from a suitably matched donor.

When the blood stem cells are collected from the patient who ultimately receives the blood stem cell transplant, that's called an autologous bone marrow transplant, and when those blood stem cells are collected from a suitably matched donor, that is called an allogeneic bone marrow or blood stem cell transplant.

Both of those options are available at Johns Hopkins.

In addition, we would complete the transplant using any one of the stem cell sources.

So we can collect stem cells from the bone marrow.

We can collect stem cells from the peripheral blood.

Or we can collect blood stem cells from the placenta or the cord blood.

We also use a variety of what are called conditioning regimens.

Myeloablative, or full intensity, conditioning regimens are generally high-dose chemotherapy and/or radiation, but we can also offer blood stem cell transplants, following what are called reduced-intensity conditioning regimens, and they are generally a bit more gentle, in terms of the complications that are seen after the blood stem cell transplant procedure, and can be offered to patients who might not have malignant disorders.

That gets us to the final types of transplants.

So we offer transplants to patients who have malignant disorders, including leukemia and lymphoma.

We also offer transplants to patients whose bone marrow is no longer working.

They may have something called bone marrow failure syndromes, aplastic anemia, for example.

We also offer blood stem cell transplants to patients with hemoglobinopathies.

Their bone marrow may be functioning for the most part, but in essence, making defective products.

And then finally, we also offer blood stem cell transplants to patients whose immune system may be dysfunctional.

Remember, these blood stem cells are going to give rise to all of the other cells in the blood and immune system, so if you have an immunologic disorder, a deficiency, or perhaps an immune disorder where the immune system is overactive, we can also offer life-saving, certainly life-changing, blood stem cell transplant procedures to those individuals, as well.

(reflective music) I think the biggest advance, not only at Johns Hopkins, but likely in the entire world today, is the use of half-matched related donor transplants.

So heretofore the approach to a blood stem cell transplant, if the donor was gonna be someone other than self, a suitably matched donor, that donor needed to be what is called HLA-matched.

Not everyone has an HLA-matched sibling.

In fact, only about 25 or 30% of patients may have an HLA-matched family member.

If those patients don't have a suitably matched family member, there are unrelated donor registries, both using adults and also using cord blood that also can be tapped into.

However, not everyone has a suitably matched unrelated donor.

So the ability to actually use a half-matched donor in a safe, effective, and transportable way, has not only been an innovation at Johns Hopkins, but this has changed the way we have done blood stem cell transplants across the globe over the last five to 10 years.

And I think that, without question, is not only the biggest advance made at Johns Hopkins recently, but again, really one of the bigger advances made throughout the world in the last decade or so.

The most important aspect of using a half-matched donor, half-matched related donor, is to be able to do this in a

way that's safe and effective, and in fact, doesn't take much manipulation of the bone marrow graft.

Using half-matched donors, in and of themselves, is not new.

People have been doing them for decades.

However, results have not been optimal whatsoever.

It turns out moving a very commonly used chemotherapeutic agent called Cytoxan, two doses of this medicine to the post-transplant period, something we refer to as post-transplant Cytoxan, has allowed us to use these half-matched related donors in a way that, again, is safe, effective, and essentially allows everyone in need, just about everyone in need, of a bone marrow transplant to have a suitably matched donor.

The most important thing is, this is relatively inexpensive and is transportable.

What I mean is you don't need fancy cell processing.

You don't need to be, necessarily, in a tremendously advanced center.

You can take this innovation and really apply it throughout the entire globe.

So now we're doing these transplants in eastern Europe, in Latin America, in southeast Asia.

So this, again, has enabled us to break down the traditional barriers to donor availability, not just here at Hopkins, but across the planet.

(reflective music) So the way half-matched transplant is influencing patient care is really predicated on, now, we have broken down donor barriers.

So now, if you don't have a suitably matched, traditionally suitably matched, related donor, or a traditionally suitably matched unrelated donor, you still now have a donor for a blood stem cell transplant.

So it is not unusual for us to see, on YouTube, or on social media, parents pleading for a match for their son or daughter, and I love to look at them, or reach out to them, and say, "Look in the mirror.

"You are the potential match." Because a mother or father is, in fact, biologically going to be half-matched to their son or daughter.

So if you're young, generally less than 50 years of age, and you're healthy, you can, in fact, be that suitable donor for a child in need.

And I think, by far, that has now allowed us to expand blood stem cell transplantation to patients, again, not only who have leukemia, lymphoma, but they may have non-malignant disorders, again, aplastic anemia, where the bone marrow shuts down, hemoglobinopathies, sickle cell disease, for example, beta thalassemia.

The blood stem cells come to work.

They make defective products.

Now we can offer a life-saving blood stem cell transplant to almost all of these patients who are in need, and that is where Hopkins is moving the field forward.

(reflective music) So this is an exciting time at the Children's Center and at the Sidney Kimmel Cancer Center.

We are now using our half-matched reduced-intensity platforms to transplant patients across the scope of malignant and non-malignant diseases, and in doing so, we are working to break traditional barriers to donor availability.

In addition, we're doing cutting-edge bench-to-bedside research to make the transplant procedures safer.

We want to reduce, prevent, or be able to treat potentially life threatening complications that can sometimes associate with blood stem cell transplants, whether you're receiving those blood stem cells from yourself, autologous bone marrow transplant, or from a suitably matched donor, allogeneic bone marrow transplant.

In addition, we're working, again, in a bench-to-bedside manner to figure out ways to help train this new donor immune system to effectively recognize and eradicate residual host leukemia or tumor.

This is a very well-known entity called graft-versus-leukemia or graft-versus-tumor effects.

So we are working diligently to figure out ways to optimize graft-versus-leukemia effects and minimize innocent bystander casualties, something known as graft-versus-host disease.

Finally, we're also working with patients, excuse me, with our colleagues, in stem cell biology to be able to look at non-blood stem cells and look at their immune modulatory capacity, and regenerative capacity, to see if we can expedite healing of certain complications after the blood stem cell transplant procedure.

So that is the mission that we're bringing forth in the pediatric blood and marrow transplant program.

We work hand-in-hand with our colleagues in internal medicine, and I think that, together, is really a force to be reckoned with, not only here in the US, but globally as well.

(reflective music) (children's laughter)