

INTERVIEW

The Economics of Cord Blood Banking



Michael Boo serves as a business consultant to the healthcare industry, particularly in the areas of blood and marrow transplantation. He is the former Chief Strategy Officer of the National Donor Program/Be The Match (NMDP), the national registry for the USA. He joined the NMDP in 2001. His contributions while at NMDP had included the redesign and implementation of a new strategic planning process that has helped guide substantial growth at NMDP, guide the development of the Center for Cord Blood in 2003, and expand NMDP growth in cord blood in the USA from 15% of the market to over 90%. Boo has identified and developed new products and services that have had significant bottom-line revenue impact and developed new relationships within the extensive NMDP network of national and international partners that have improved access to cell sources and markets worldwide. He has provided leadership and oversight for legislative activities, has helped establish a new federal funding program for cord blood inventory growth, which has provided more than \$100 million to date, and pursued other strategies to continue and expand government funding sources. More recently, he has led a number of initiatives to address deficiencies in reimbursement for transplant related-costs by both the public and private payers.

Q You recently worked as the Chief Strategy Officer at the National Marrow Donor Program (NMDP). Can you tell us a bit more about the NMDP's function and how it supports cord blood banks?

The NMDP operates the Be The Match registry of unrelated hematopoietic cell donors and umbilical cord blood units in the USA. It has the world's largest hematopoietic cell registry with around 225,000 listed cord blood units. It provides this service under contract with the Health Research and Services Administration, a department of the Health

and Human Services Department of the federal government.

Under that contract, it is the NMDP's responsibility to develop and maintain a registry of publicly available cord blood units that would be searchable by transplant centers on behalf of patients interested on an unrelated cord blood donor transplant. The NMDP's role is to develop and maintain that list, promote awareness of cord blood as a source of cells for transplant and to increase donation to public cord blood banks, including assuring that parents had good information to make the decision to donate. So those were our broad mandates. As the NMDP's Chief Strategy Officer, I was responsible for overseeing the development of the relationship between the NMDP and the cord blood banks among other things.

Q Based on your experience at the NMDP and work elsewhere, what trends have you seen in terms of cord blood banking in the last 10 years?

Over the last 10 years we've seen significant change in the cord blood banking field. From an unrelated donor standpoint in particular, over the last 10 years, there has been significant increase in the adoption of cord blood as a source of cells for unrelated donor transplant to deal with hematopoietic cell reconstitution.

Cord blood was used as an alternative and complementary source of cells to other existing sources, marrow and peripheral blood stem cells from adults. The last 5 years have seen a levelling off and even a reduction in the use of cord blood as a source of cells for that same purpose. The market trend has seen a significant increase and then slowly a reduction in the use of cord blood for hematopoietic cell transplantation.

Also, during that period of time we've seen the emergence of a licencing scheme by the FDA to licence this as a biologic that was instituted in 2011. And is still being rolled out. However, not all banks are licenced at this point, but licencing continues to be a requirement. So we've seen the increasing regulation of cord blood in the USA. Other countries have begun to regulate cord blood and other cellular sources as well.

Q What are the major challenges that the cord blood industry faces?

The greatest challenge to cord blood in the short term is whether and to what extent it will continue to be used as a source of cells for transplantation.

The reduction in its use has been attributable to a number of factors. One is the cost of the cord blood unit itself compared to other sources of cells. Some centers have also faced difficulties in managing the transplant because it takes longer to engraft, and there tends to be higher level of complications to manage. These factors have contributed to its slower adoption and perhaps the decline is due to alternate sources of cells including the emergence of haploidentical transplant as another source of cells when you don't have a fully matched donor. These factors continue to represent challenges to the cord blood industry in the near term.

In the long-term the question for cord blood is first of all within transplant, can some of these issues in cost and time to engraftment be managed successfully because there is still therapeutic value to cord blood. So can other issues be managed such as through expansion technologies and other adjunct therapies being developed to allow cord blood to be used to address some of these problems we're seeing. Examples are the technologies being developed by Gamida Cell for example, and the product from Nohla, of Colleen Delaney's lab, as examples of some of these efforts to make use of cord blood more effectively for its current use.

There's also the possibility of using cord blood as a source of cells for other regenerative medicine. Several interesting studies have been done in a variety of diseases, where cord blood was used as a cell source for instance, it to treat heart disease, orthopaedic conditions, etc. Dr Joanne Kurtzberg has done a lot of work in cerebral palsy and traumatic brain injury.

So are these opportunities going to be available for cord blood? Does cord blood provide a desirable source of cells for the new cellular therapies? The question is still there. I think there's some interesting science, but I don't think it's been proven out completely that cord blood will be the best source of cells. But that would be one of the things that cord blood bankers will be looking forward to, seeing it happen.

Q What are the key differences between public, private and hybrid banking models in terms of economic viability and therapeutic value?

From an economic standpoint, certainly these are two different business models. Public banking requires significant initial investment to develop the inventory. Then with the banks having to wait until the need is presented, and without a large inventory the need can't be met. So there's significant capital investment required to develop that inventory over time. Without demand increasing, and in fact with it decreasing, continuing investment in that inventory is a challenge.

Private banks obviously have a different model. They are paid as they go. The families pay for the initial collection and storage, and pay for on-going maintenance of that cord blood unit. So their economic model is much more secure as long as they continue to be some potential for self-use or family use of these stored cord blood units. That again is still yet to be determined. To a large degree private banks still depend on the promise of the use of these units rather than the demonstrated use. And so private banks are still waiting for the science to really emerge that cell storage makes sense over other therapies into the future. But at least for the moment their economic model is somewhat self-funding until that happens.

Q What are the key strategies being tested to sustain the economic future of Public Cord Blood Banks?

There are a couple of strategies being tested. One certainly has been to be more disciplined in the selection and banking of units into

the public inventory. Some banks such as CariCord out of Colorado have adopted a very selective approach to banking public units in order to make sure the units they do bank and the investment they make in those units will result in a higher percentage likelihood of use.

So one strategy is to be more selective in what cord blood units to bank. We do have a significant inventory of smaller units so banking larger units becomes really the dominant theme.

A second approach is the emergence of these hybrid banks as some people describe them. These are where the public banks have adopted or developed a parallel private banking component to be able to offer families that are interested in banking multiple options, both public and private, through a single site. And to make better use of the current investment in infrastructure, and at the same time benefit from the cash flow that develops from a private banking model to perhaps weather some of the economic storm of public banking, and see what happens.

It's certainly much more difficult to go the other way to develop public banking off a private banking model because what you're really doing is burdening the private banking business model with the significant costs against potential in the future. We haven't seen many private banks moving to public banking, but we've certainly seen public banks expand their efforts by developing private banking to create an economic stream that will hopefully maintain them and provide a better marketing position for them.

Q What impact will haploidentical stem cell transplant potentially have on the cord blood industry?

Haploidentical stem cell transplant has shown itself to be a very easy transplant to manage. Firstly, almost everyone has haploidentical units somewhere in their family tree. Secondly, it's lower cost than an unrelated donor product. Ease of management of the patient and rapid engraftment seem to have provided a short-term solution for patients who don't have a fully matched adult donor product. I think this has crowded our cord blood bank, because every transplant center wants as many alternatives as possible for patients. If they don't have a fully matched related donor or fully matched unrelated donor, what is left for them? It's either cord blood or haploidentical blood. For many centers, haplo is a preferred choice over cord blood because it is easier to adopt.

The trend we've observed is that, more centres over time have added haploidentical protocol year over year, over the last 5 years. And fewer transplant centers are doing cord blood year over year. There seems to be some relationship between the adoption of haploidentical and diminishing use of cord, although the relation isn't clear when we look on a center-by-center basis.

Many believe that the jury remains out in terms of the long-term durability of haploidentical grafts. I think we're now starting to get into 3- and 4-year follow-ups, so maybe we'll hear more about how durable it is. But we had noted the continuing relapse in haploidentical 3 and 4 years out and that is something you don't see in cord blood or matched adult donor transplants.

So is that relapse in the haploidentical side going to tip the scale back to cord blood? Will the long-term concerns with haploidentical will outweigh the short-term benefits of providing an immediate and readily available transplant for patients in need?

Q Have you observed any regional differences in cord blood banking, such as areas with higher than average growth potential?

In the USA, we don't see a regional difference. Those transplant centers that have had a strong commitment to cord blood continue with that commitment and continue to do research in cord blood. Most of those centers have also adopted a haploidentical protocol as well.

Centres that have a weak commitment to cord blood, doning 1 or 2 a year, are the ones moving away from cord blood entirely. So the trend is not regional, but varies on a center-by-center basis.

A similar trend is seen in Europe. We see a drop in use of cord blood as source of cells. Some countries that have adopted it in a big way, such as France seem to be reducing use. Italy has always had both haploidentical and cord blood, but seems cord blood is dropping off and haploidentical transplant seems to continue to advance as a source of cells. The UK is a recent addition to cord blood in a big way and they too seem to be moderating, although it's not clear what will happen there.

Taken together, we see differences between countries and overall there's a drop in cord blood banking in Europe. Only in Japan and some other Asian Pacific rim countries do we see continuing growth in cord blood use.

Q How do you see the cord blood industry evolving in the next 5–10 years?

Iwould anticipate some consolidation in the public side. I think even if there is some resurgence in the use of cord blood for transplant, it's probably going to be still modest growth and not significant growth. In the end we just don't need so many banks storing cord blood units, but a more selective collection and banking.

Likewise in the private side, I think there are still more and more entries into the private banking. But the big players have dominated the marketplace now for the last few years and it will be harder for new entries.

Again, I think private banks and public banks are waiting to see whether cord blood can be a source of cells for some of these other diseases currently being studied, whether it's cerebral palsy we talked about, autism, diabetes. If it does, that would open more opportunities to both public and private banks and right now we have to 'wait and see' for research to develop.

AFFILIATION

Michael Boo

Business Consultant for the Cord Blood Industry
(Former Chief Strategy Officer at NMDP).

