

Challenges faced by the
Cord Blood sector

Top 10 experts opinions

- **Who is the most influential figure in cord blood around the world?**
- **What do you think has been the biggest achievement within the cord blood sector?**
- **Which is the most important development in cord blood research?**
- **What is the biggest challenge to overcome in the use of cord blood as a source of stem cells?**

We asked 10 leading experts in the cord blood world what they thought about the current state of the industry, what the greatest achievements in research were, what the biggest challenges were and who had done the most to advance the field of cord blood.

The cord blood banking industry is one of the fastest growing industries in the world, with cord blood banks increasing by 21 fold in less than 10 years, but recent challenges have meant that there are still issues that need to be addressed. High costs of units due to expensive storage and extraction, a lack of available units, and a plateau in the development of regenerative applications mean that a meeting between key players in this area is greatly needed.

Today cord blood is more relevant to the regenerative medicine community than ever. There is a huge amount of research currently happening in this area to utilise cord blood as well as other cells and tissues in increasingly wider areas of medicine. New business models have recently been brought into practice as the cord blood sector has developed and moved away from traditional models to increase collaboration and benefit the wider population. Leaps in innovation also mean more cost effective extraction, collection, bioprocessing, and storage.

In this rapidly evolving market it is important to stay up to speed with where the industry is headed and what your competitors are doing. That is why we bring you ten expert opinions from pioneers working in different areas in the cord blood sector.

So, get comfortable and enjoy finding out what the greatest achievements and challenges are facing the industry today.

Exclusive to Bioinformant subscribers, we have updated this eBook especially to include an additional interview from Dr Jaap Boelens of UMC Utrecht, pioneer of 2 recent novel therapeutic applications for Cord Bloods and an updated interview from Dr Husein Salem, Founder & CEO of Precious Cells one of the most pioneering cord blood banks in the UK.

Do you have anything to add? If so, I'd love to hear from you!

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Michael Boo, Chief Strategy Officer, National Marrow Donor Program

What do you think has been the biggest achievement within the cord blood sector?

Beyond the demonstration that umbilical cord blood (UCB) is a source of cells that can reconstitute the immune system reliably and safely, the biggest achievement has been the use of UCB to extend access to populations that would not otherwise have access to a life-saving transplant. Over 30% of cord blood transplants are for minority patients in the United States, compared to 14% of adult donor transplants, utilize a UCB as the primary cell source. The distribution of UCB by racial and ethnic minorities is consistent with the US population as a whole.

In your opinion, which is the most important development in cord blood research?

The most important development today is the focus on the techniques and technology to address delayed to engraftment and immune cell recovery and the attendant mortality and cost associated with the delay. Great work is being done across transplant centers in many countries to investigate expansion of CBUs, supportive infusion of haplo-identical grafts, and similar ideas. With UCB use stagnate or in decline, these developments may reverse this trend by significantly improving outcomes for patients.

Who do you think has done the most ground breaking work using cord blood?

There are too many excellent researchers and clinicians to venture to say which one has done the most ground breaking work. This field is notable for the work of many people building on those that have come before them to continue to improve and extend this therapy. Certainly, it is worth noting the efforts of Hal Broxmeyer and Eliane Gluckman to manage the first cord blood transplant, the pioneering work of Pablo Rubinstein defining the science of cord blood banking, and the continuing work of clinicians like Joanne Kurtzberg and John Wagner to extend the use of UCB to new indications. But there are many more

people involved in the field worthy of note, including those engaged in the current exploration of expansion technology which will further evolve this field.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

The biggest challenge today is the relative clinical benefits of UCB transplantation compared to the cost. UCB transplantation is relatively costly compared to the alternatives, and in the case of haplo-identical transplants, significantly more costly, at least in the short term. This cost concern extends from the cost of the graft itself to the cost of managing the patient through engraftment and neutrophil recovery. More resources are typically dedicated to the first 100 days of care for UCB patient than transplants involving other cell sources. It may be that the relative advantages of using UCB are offset by these costs and related complications, which is a concern in an increasingly cost conscious health care world. There is a great need for comparatively effectiveness research to determine the relative value of UCB transplantation compared to the alternatives over time. And more work needs to be done to reduce time to engraftment and the durability of the UCB transplant in any event.

Cristina Navarrete, Director, N.H.S.B.T

What do you think has been the biggest achievement within the cord blood sector?

- The demonstration that Cord Blood is a suitable alternative source of haematopoietic stem cell for transplantation in patients with haematological, immunological or metabolic genetic disorders and is associated with milder aGVHD.
- Establishment of Cord Blood banks worldwide.
- Developments and implementation of Standards and Accreditation programmes for Cord Blood Banking and transplantation.

In your opinion, which is the most important development in cord blood research?

- Potential of cord derived mesenchymal stem cells in regenerative medicine and immunotherapy protocols.
- Identification of protocols for expanding the cord blood stem cells.
- Identification and expansion of other regulatory or effector cell populations present in cord blood.

Who do you think has done the most ground breaking work using cord blood?

John Wagner, Hal E. Broxmayer, Elaine Gluckman, Pablo Rubinstein, Joanne Kurtzberg.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

For transplantation: the limited dose collected and cost. For immunotherapy and Regenerative Medicine, the development of clinically acceptable protocols for the use of the differentiated or expanded cells i.e. under GMP conditions.

Carlos Moreno, General Manager, BioCord

What do you think has been the biggest achievement within the cord blood sector?

To generalise the banking of cord blood units all over the world, for public and private uses.

In your opinion, which is the most important development in cord blood research?

Hematopoietic stem cell expansion. Also, mesenchymal stem cell isolation and expansion.

Who do you think has done the most ground breaking work using cord blood?

Prof. Hal Broxmeyer, developed cryobiology around Cord Blood.
Dr. Elaine Gluckman, did the first transplant using a Cord Blood unit.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Hematopoietic stem cells expansion before transplantation and enhancing immune reconstitution after transplantation.

Charis Ober, Founder, Save the Cord Foundation

What do you think has been the biggest achievement within the cord blood sector in the last 12 months?

The possibility of using autologous cord blood stem cells to treat autism.

In your opinion, which is the most important development in cord blood research in the last 2 years?

Expansion of cells is one of the most difficult challenges left to tackle.

Who do you think has done the most ground breaking work using cord blood in the last 2 years?

Dr. Curtis Cetrulo: Cadaver limb transplantation.

I heard Dr. Cetrulo speak at a regenerative medicine meeting and was in awe of the work he is doing to advance cadaver limb transplant for amputees. Dr. Curtis Cetrulo, a plastic surgeon, transplant expert and stem cell researcher is truly making a difference and changing transplant medicine with his surgical allotransplantation applications for using cadaver limbs to replace amputated limbs. Dr. Cetrulo's stem cell research and innovative transplant surgical techniques are revolutionizing the world of limb transplant surgery and making brighter futures for patients who had previously had only prosthetic arm, leg and finger options.

Dr. Curtis Cetrulo is the Senior Investigator and Head of the Vascularized Composite Tissue Allotransplantation Laboratory at the Transplantation Biology Research Center, Massachusetts General Hospital graduated from Stanford University in 1992 and Tufts University School of Medicine in 1999.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Education. This is critical! We need to not only inform expectant parents about the medical value of umbilical cord blood, but we also need to educate the public so they also understand the need to expand umbilical cord blood collection, education and research. In my home state of Arizona we are connecting public donation, education with research and business at a state level, we want to create a sustainable cellular and science business and education platform where cellular technology and conservation is understood and respected part of our children's and citizens health culture. The next generation of young men and women are the key. We need to teach them the importance of saving these precious cells and that CBSC's are the future of health and science.

Chris Goodman, CEO, Virgin Health Bank

What do you think has been the biggest achievement within the cord blood sector?

Raising awareness to stem cells as a potential lifesaving / life enhancing option.

In your opinion, which is the most important development in cord blood research?

IPSCs cells.

Who do you think has done the most ground breaking work using cord blood?

Joanne Kurtzberg, Duke University.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Stable clonal expansion beyond 15x without differentiation for adult and multiple transplants from a single unit.

Gesine Kögler, Director, Jose Carreras
Blood Bank Düsseldorf

What do you think has been the biggest achievement within the cord blood sector in the last 12 months?

That public and private banks work together at the Cord Blood Association.

In your opinion, which is the most important development in cord blood research in the last 2 years?

Phase I/II studies in regenerative medicine (controlled clinical trial, double blind studies).

Who do you think has done the most ground breaking work using cord blood in the last 2 years?

Joanne Kurtzberg.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Convince transplant physician's that Cord Blood is better; reduce the price of allo-Cord Blood (double Cord Blood units). Do serious research in animals as a prerequisite for studies in humans!

Kourosh Saeb-Parsy, University
Lecturer & Consultant Transplant
Surgeon, Cambridge University
Hospitals NHS Trust

What do you think has been the biggest achievement within the field of cord blood research in the last 12 months?

Discovery of UM171 molecule as a possible method for expansion of UCB.

In your opinion, which is the most important development in cord blood research in the last 2 years?

Co-transplantation with MSCs to enhance engraftment.

Who do you think has done the most ground breaking work using cord blood in the last 2 years?

Virgin Health Bank, by introducing a sustainable new model of 'public' cord blood banking, in which cord blood is available to clinicians free of charge.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Cost, availability and stem cell dose.

Mike Watts, Director Cellular Therapy, UCLH

What do you think has been the biggest achievement within the cord blood sector in the last 12 months?

The continued expansion of cord banks (about 500 Worldwide) and centralisation of registries such the Anthony Nolan representing the whole of the UK.

In your opinion, which is the most important development in cord blood research in the last 2 years?

Too early to say which application(s) will prove the most fruitful but the number of clinical trials registered for regenerative applications demonstrates the importance of Cord Banks as a bio-repository of cells extending their use beyond transplantation applications alone.

Who do you think has done the most ground breaking work using cord blood in the last 2 years?

Many contenders, but the basic stem cell biology work of Delany *et al* is of particular interest and has translated to clinical efficacy eg Delaney et al 2013 Biol Blood Marrow Transplant 19 (suppl 1) S74-78

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

As ever, early engraftment delay and low cell numbers for adult use. In vitro expansion, haplo+cord are expensive site-specific options.

Improving haplo transplant outcome seems to be the greatest factor curtailing the selection of cord blood where a matched donor is not available. The latest EMBT report (2013) from Passweg *et al* 2015 BMT (on line) for example shows the trend in HSCT donor source selection reported for transplants from 2010 to 2013 as follows: Haplo-identical relatives 801 to 1571 (+96%), cord blood 789 to 666 (-16%).

Dr. Mrinalini Chaturvedi, President (Medical Affairs, R&D), of Cryobanks International India

What do you think has been the biggest achievement within the cord blood sector?

Biggest achievement is that cord blood transplants as an alternative to bone marrow transplants have successfully saved many lives, and have become a standard of care. Cord Blood Banking has set high standards of quality assurance in cellular therapy arena.

A medical waste can be used as a life-saving product because of availability of cord blood banking services.

In your opinion, which is the most important development in cord blood research?

Most important development is for additional uses of stored and freshly collected cord blood cells such as generating iPSC from cord blood to accelerate translational research for therapeutic applications. Banks of iPSC customized for large-scale screening and therapeutic purposes can be developed. Secondly if it would be possible to provide family banked cord blood units for wider use beyond family to enhance utility of large number of private cord blood units banked.

Who do you think has done the most ground breaking work using cord blood?

Ex vivo expansion of the HSCs in a unit of UCB to obtain more cells for long-term multi-lineage engraftment.

Secondly successful clinical use of UCB for adults, pioneered at the University of Minnesota, using double cord blood units to increase the combined cell dose and substantially improved time-to engraftment for adults compared with the use of a single UCB unit.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Biggest challenge is to get a good volume and eligible cord blood unit with high dose of stem cells so that it has a high possibility of utilisation in cord blood transplants. The other big challenge is the negativity surrounding private banking as well as lack of awareness.

Dr Sergio Queral Giner, Director of the
Haematopoietic Progenitor Cell unit
and Cord Blood Bank, Banc Sang i
Teixits, Barcelona

What do you think has been the biggest achievement within the cord blood sector?

Being an evidence-based-medical therapy after more than 40,000 procedures performed worldwide.

In your opinion, which is the most important development in cord blood research?

Proven the enhanced graft vs. leukemia effect on cord blood transplant.

Who do you think has done the most ground breaking work using cord blood?

Double cord blood transplantation.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Improving immune reconstitution after cord blood transplantation.

Calvin Cole, President & CEO, CARICORD

In your opinion, which is the most important development in cord blood research?

FDA Drug Quality units that can be used in this development to speed along the FDA Trials.

Who do you think has done the most ground breaking work using cord blood?

Dr. Pablo Rubinstein at the National Cord Blood Program for the first FDA Drug License.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

A larger inventory of high quality units is needed to give transplant centres more of a selection.

Dr Hussein Salem, Founder & CEO, Precious Cells

What do you think has been the biggest achievement within the cord blood sector?

I believe opening up frameworks for use of cord blood across wider patient base has been the most significant achievement. Both within blood/immune uses to open up to older patients and by double/triple transplants and possibly more importantly its use in non-blood related clinical trials such as Autism, CP and DM has propelled interest in the sector.

In your opinion, which is the most important development in cord blood research?

Legislative changes to open up cord blood use as a biologic and biobetter.

Who do you think has done the most ground breaking work using cord blood?

It's been a global collaborative effort, in the UK, Precious Cells Group has done more for biobanking than anyone, in the use, groups like CBR , Cariocord, Viacord and in China, the China Cord Blood Group are leaders and innovators.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Regulators and the regulatory landscape needs to catch up and re-invent the way they operate as they are in danger of falling behind.

Dr Jaap Jan Boelens, Pediatric
Oncologist / Immunologist, Division
Laboratory and Pharmacy, Blood and
Marrow Transplantation Program,
University Medical Center Utrecht

What do you think has been the biggest achievement within the cord blood sector?

Wide applicability and availability of cord blood as alternative cell source in Hematopoietic Cell Transplantation.

In your opinion, which is the most important development in cord blood research?

Expansion protocol (to overcome long neutropenia).

Who do you think has done the most ground breaking work using cord blood?

Joanne Kurtzberg.

What do you see as the biggest challenge to overcome in the use of cord blood as a source of stem cells?

Get single cord blood units available for all patients who need an allo-HCT.

It seems as though there are still many milestones to overcome if cord blood is to be a viable, cost effective option for transfusion and other stem cell therapies in the future. Cord blood banks need to up their game to make a return on their business. From intelligent tissue typing to improving quality of units and educating physicians there are many areas where cord blood banking needs work. This source of stem cells is far too valuable to overlook however, and if we work together we can overcome these challenges.

You've heard what the experts think, now join them! They will all be at the [Cord Blood World Europe Congress](#).

Aiming to bring together public and private banks, clinicians, researchers, and solution providers to tackle the challenges and opportunities facing this field of regenerative medicine. On **20th - 21st May in London** we will do something amazing.

Come and join us and make a difference, show yourself as an industry leader, stay ahead of your competitors, make new collaborations and learn how to make your cord blood bank a success.

You can't afford to miss it.

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