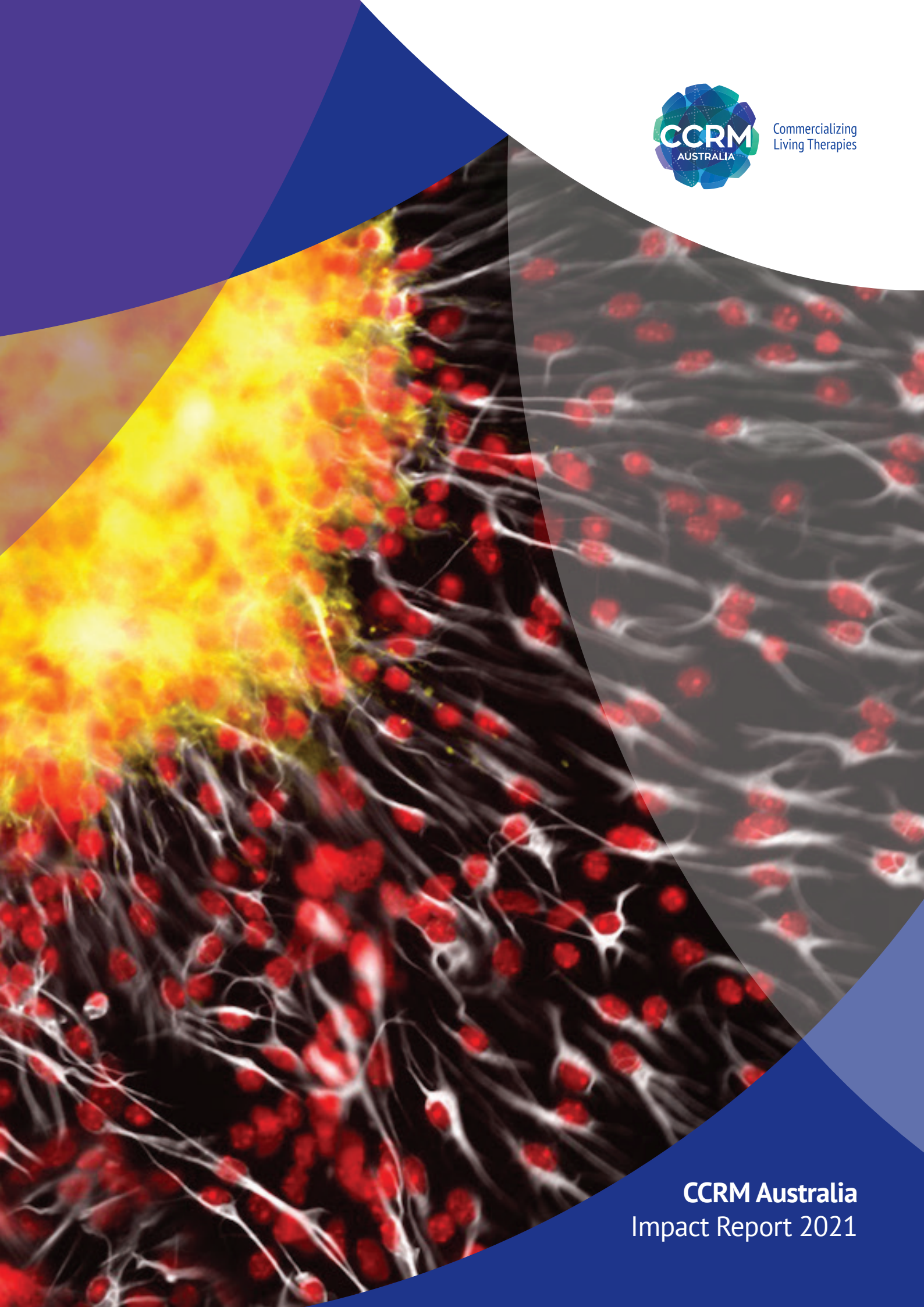


CCRM AUSTRALIA

Commercializing
Living Therapies

CCRM Australia
Impact Report 2021

CONTENTS

Front cover image: "Cell Supernova" by Danielle Spice. Shown here are P19 embryonal carcinoma derived astrocytes migrating away from an embryoid body. Around the embryoid body neurons are also visible.

COLLABORATIONS

Founding Organisations

Monash University, CSIRO, CCRM, cell therapies, ARMI Australian Regenerative Medicine Institute, St Vincent's Hospital Melbourne

Spotlight Partners

Griffith—Hack, Opyl, Eureka Technology Innovation & Investment, 红日药业集团 Chase Sun, ARC CTET, BioPlatforms Australia

Ecosystem Partners

National Stem Cell Foundation of Australia, aimss Australian Institute for Musculoskeletal Science, Hudson Institute of Medical Research, Therapeutic Innovation Australia, BioLink, indee labs, BioCurate, IMNIS Industry Mentoring Network in STEM, Aeterna Health Services, Centre for Eye Research Australia, Bond University, ortho-cell advancing tissue repair & regeneration, cynata therapeutics, vivazome, StemCore, Australian Red Cross Blood Service, Co-Labs, NSW StemCell Network, SVI St Vincent's Institute Medical Research, QIMR Berghofer Medical Research Institute, WAHTN Western Australian Health Translation Network, QUT Institute of Health and Biomedical Innovation, The Florey, World Courier AmerisourceBergen, TRI Translational Research Institute Australia, Ear Science Institute Australia, neuroscience trials australia, Tessara Therapeutics, ACCTERM, Medtech Actuator, Invetech, Kobold Ventures, regeneus, excpharm, Monash Institute of Medical Engineering, ReNerve, AusBiotech Australia's Biotechnology Organisation, Life Sciences Queensland, BioMelbourne Network Progressing Biotechnology, CARTherics, endexome a regenerative medicine corporation

Government Support

Victoria State Government, Supported by MTPConnect MedTech and Pharma Growth Centre

ACTIVITIES AND ACHIEVEMENTS

CCRM Australia accelerates the commercialisation of regenerative medicine therapies and related technologies. We do this by providing specialist expertise, funding opportunities and connections between industry, clinicians, researchers, and other key stakeholders.

TRAINING PROGRAMS:

CCRM Australia Internship

Vishnu Vijai Vijayakumar (University of Melbourne)
Bhavi Kadakia (Monash University)
Dharvi Patel (Monash University)
Mahmuda Khatun (Monash University)
Harmanpreet (Monash University)
James Tran (RMIT University)

First Half of 2021:
Aashima Godara (Monash University)
Bobby Halim (Monash University)
Yamuneshwaran Ganapathy Kamal (Monash University)

Summer by Design Workshop
Over 13 applications were received for the Summer by Design 2020 Workshop. James Tran (RMIT University) and Margeaux Hodgson-Garms (Monash University) were selected but was unfortunately cancelled due to COVID19 pandemic.

Webinars
CCRM Australia partnered with BioMelbourne Network to co-host 6 BioBusiness Insights webinars that showcase presentations from both local and international speakers aimed at driving business success, uniting the industry across research, translation and commercialisation, and progressing trade and export activities.

CCRM Australia continues to engage with the Australian regenerative medicine sector and currently has:

6 Founding member organisations providing financial and support services to CCRM Australia

41 Australian partners representing over 50 organisations that include providers of specialised research, academic, health, clinical trials and advisory services and related facilities

3 peak bodies as partner organisations representing over 3,000 members

4 funders/ sources of capital for investment

2 government agency supporters

COMMUNICATIONS PLATFORM PERFORMANCE:

TWITTER
1176 followers (an increase of 8.1% from the 1087 followers as at the end of July 2020)

LINKEDIN
632 followers (an increase of 33.8% from the 472 followers as at the end of July 2020)

IN THE 2020–21 YEAR THE CCRM AUSTRALIA TEAM HAS:

Directly liaised with 80 different companies

Directly liaised with 24 different research institutes and organisations

Directly supported companies from 7 different countries to access Australian resources

Directly liaised with 9 local and international government departments and agencies

Conducted 116 discussion meetings with Industry

Represented CCRM Australia at 11 events hosted by other organisations and various meetings

Undertaken 10 promotional roadshows nationally and internationally

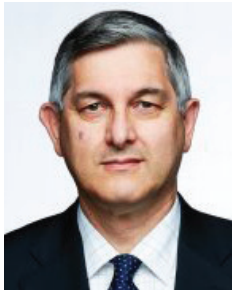
Created **4** jobs

Created **9** student intern placements

Organised 4 Speaking Engagements involving CCRM Australia representatives

Progressed 3 Industry Based Research Projects

CCRM AUSTRALIA CEO REPORT



The last 12 months have seen tremendous changes in many aspects of our working lives. Yet, notwithstanding the hardship caused by the coronavirus pandemic, CCRM Australia continues to develop as an integral component of Australia's regenerative medicine sector.

Our instincts were correct that the CCRM approach was needed to develop the Australian sector, leading to increased translation and commercialisation. As a result, there has been an excellent demand for our services. We have helped Australian organisations and researchers facilitate access to research infrastructure, secure funding for research and development and supported their international expansion objectives. We are also pleased to say that we have helped international researchers and organisations to identify collaborations with local companies and researchers. In fact, as of the end of the 2020-21 financial year, we have been able to help a total of 71 organisations and research groups meet their objectives.

With this activity level, we have the confidence to say that we now have a good understanding of the Australian sector and how it can be further developed. We are fortunate to have taken inquiries from investors and pharmaceutical companies to co-develop programs that help identify suitable investment opportunities in Australia.

Of great interest is the manufacturing facilities established and now managed in Toronto by CCRM. There are outstanding advantages in looking at this model closely and replicating it in Australia. CCRM Australia is pleased to be able to act as the local catalyst for this. We are in discussions with several Australian states regarding their manufacturing requirements and hope to play a pivotal role in their decision making.

It has also been agreed by CCRM and other hubs currently being developed as the CCRM global network that we take the SuperPitch program globally, incorporating new investors and technologies into the process. This will introduce additional opportunities to Australian researchers and organisations taking part in this program.

There are great plans to expand the training program and opportunities for Australians wishing to take advantage of the opportunities presented by the sector and will soon be making some exciting announcements.

Also, on the near horizon are plans to expand into new and developing areas supporting the development of regenerative medicine related therapies, including artificial intelligence in clinical trials.

Of course, we will continue to work with other independent CCRM hubs currently under development in Scandinavia, the Netherlands, Korea and Israel.

We intend to leverage the experience and expertise of CCRM to benefit Australia, and we will also leverage Australia's excellent capability and world standing in regenerative medicine to benefit international healthcare activities.

We are thankful for the ongoing support of key stakeholders to CCRM Australia, including founding members Monash University, CSIRO, Cell Therapies Pty Ltd, the Australian Regenerative Medicine Institute and CCRM. Financial support from the Victorian state government and MTP Connect is also acknowledged and very much appreciated.

I am also grateful for the support and hard work of the small team at CCRM Australia, Dr Chih Wei Teng, Ms Heather Marriott, Dr Jack Lambshead, Dr Wade Kruger, Dr Anita Pinar (from July 2021), Mr James Tran and several student interns for their enthusiasm and dedication to CCRM Australia and the Australian regenerative medicine sector.

Silvio Tiziani
Chief Executive Officer

CCRM CANADA PRESIDENT AND CEO REPORT

The Centre for Commercialization of Regenerative Medicine is an internationally recognised Canadian, public-private partnership supporting the development of foundational technologies that accelerate the commercialisation of stem cell- and biomaterials-based products and therapies. It does this by accessing strategic funding and providing dedicated infrastructure and specialised business and scientific expertise.

As we celebrate 10 years since the founding of CCRM, we are proud of the specialised infrastructure we have built with partners, the expert team we have brought together and the impact we are making through company creation and investing in start-ups. I'm pleased with our track record of supporting 14 companies along with almost CAD$800 million in funding raised by CCRM portfolio companies. Together with our expanding suite of dedicated manufacturing facilities, CCRM is having a significant impact on growing a successful regenerative medicine ecosystem in Canada.

With continued enthusiastic financial and in-kind support of the State Government of Victoria, CSIRO, St Vincent's Melbourne, Monash University, the Australian Regenerative Medicine Institute, we can continue the good work of the Centre for Commercialisation of Regenerative Medicine (CCRM) Australia and its positive impact in the Australian regenerative medicine sector. Since its inception, CCRM Australia has taken a national approach to support the Australian sector's development and helped accelerate the commercialisation of Australian regenerative medicine products and therapies. We are proud to be a co-founding organisation for this initiative, one of several independent CCRM hubs being established to form the CCRM global network of hubs.

Congratulations to Silvio and his team for their success. I look forward to working with everyone at CCRM Australia and in the Australian regenerative medicine sector as we look towards integrating the different hubs around the world to bring these exciting new advanced therapies to the patient.

Michael May
President and Chief Executive Officer
Centre for Commercialization of Regenerative Medicine

CORPORATE MESSAGE

CCRM AUSTRALIA

CCRM Australia is an Australian not-for-profit organisation supporting the development of foundational technologies to accelerate the commercialisation of regenerative medicine products and therapies.

CCRM Australia's focus is to bridge the commercialisation gap through a network of scientists, entrepreneurs, academic institutions and industry partners and address bottlenecks in the industry.

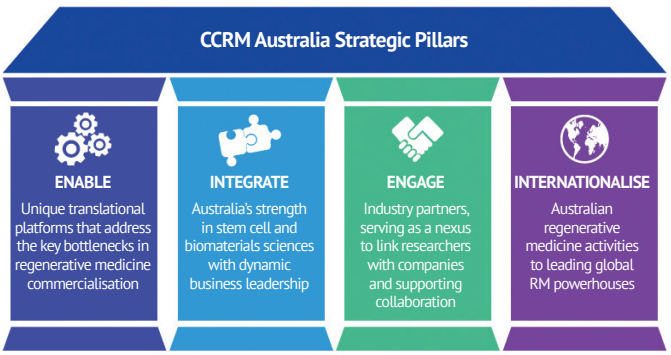
Modelled on the highly successful Centre for Commercialisation of Regenerative Medicine (CCRM) based in Canada, CCRM Australia is a leader in developing and commercialising regenerative medicine, cell and gene therapies. Drawing from the organisation's expertise and industry and capital venture networks established over a five-year period, CCRM Australia is poised to take a leading role in supporting Australia's fast developing regenerative medicine sector.

MISSION

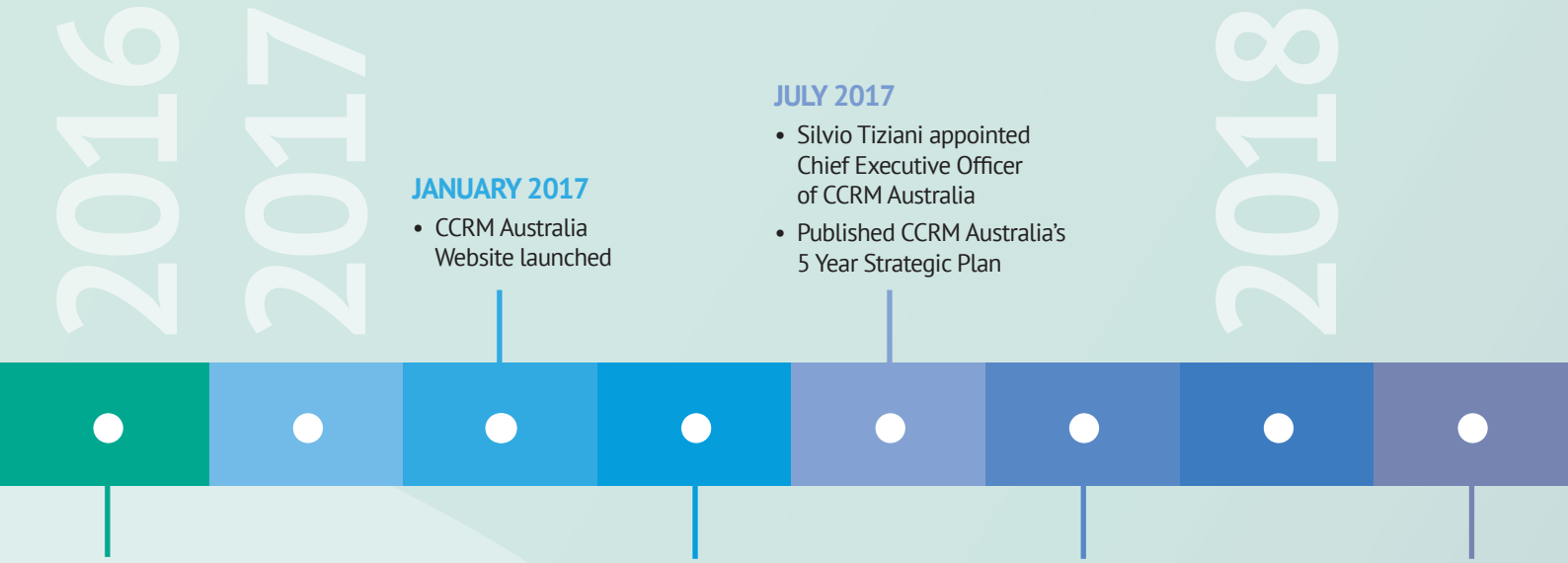
CCRM Australia is taking regenerative medicine from bench to bedside by treating and potentially curing some of the most devastating and costly diseases in the world today.

VISION

We seek to enable, integrate, engage and internationalise the Australian regenerative medicine industry.



CCRM AUSTRALIA MILESTONES



OCTOBER 2016

- CCRM Australian Initiative Announced – received funding from MTP Connect

JANUARY 2017

- CCRM Australia Website launched

MARCH 2017

- 5 year strategic plan endorsed by CCRM Australia's Steering Committee and MTPConnect

JULY 2017

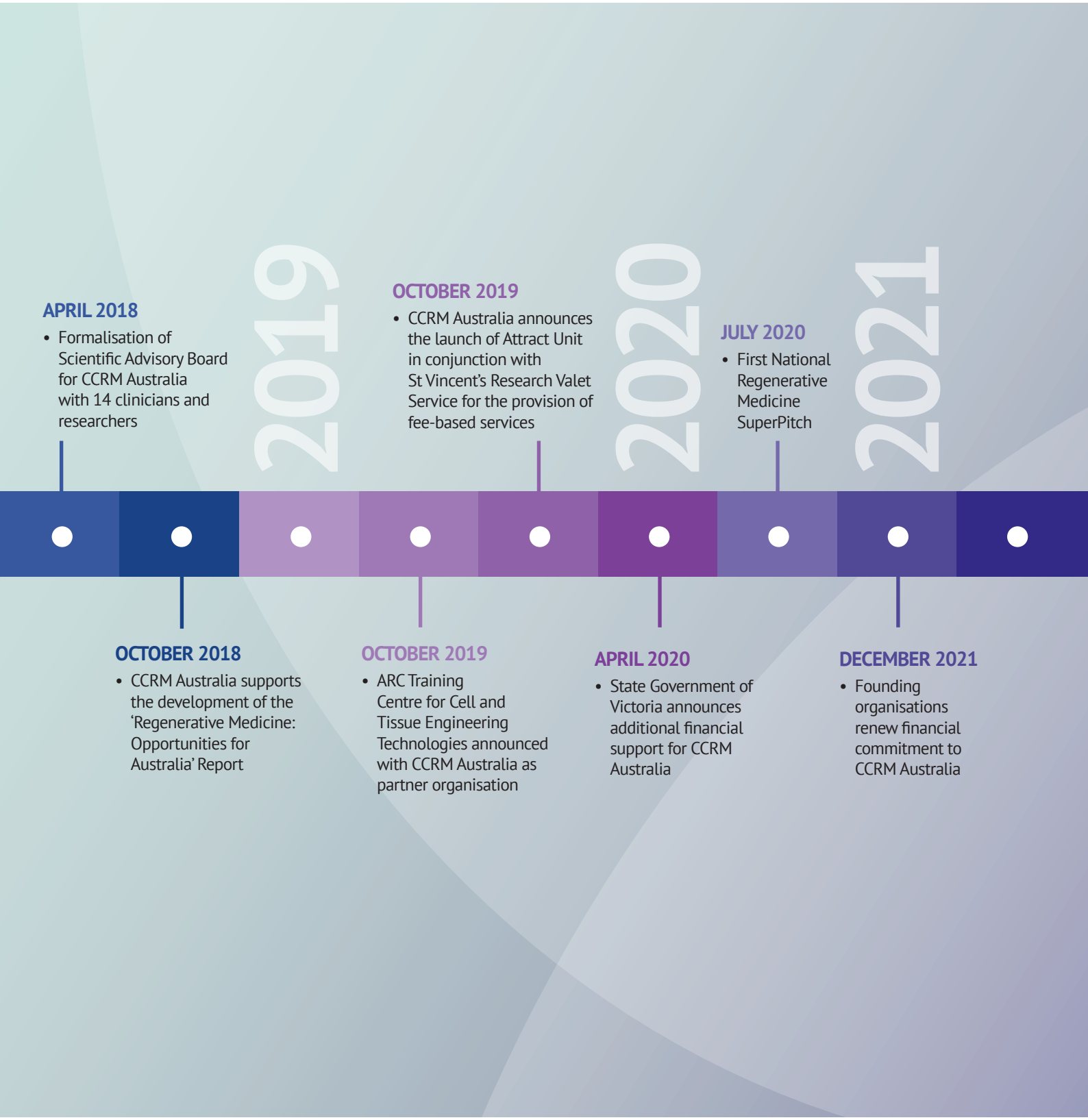
- Silvio Tiziani appointed Chief Executive Officer of CCRM Australia
- Published CCRM Australia's 5 Year Strategic Plan

OCTOBER 2017

- CCRM Australia successful in securing additional funding in MTPConnect Grant Funding Program (Round 2)

FEBRUARY 2018

- Establishment and first meeting of the CCRM Australia Council
- Establishment and first meeting of CCRM Australia's Industry Interface Committee


2019
2020
2021
APRIL 2018
• Formalisation of Scientific Advisory Board for CCRM Australia with 14 clinicians and researchers
OCTOBER 2018
• CCRM Australia supports the development of the 'Regenerative Medicine: Opportunities for Australia' Report
OCTOBER 2019
• CCRM Australia announces the launch of Attract Unit in conjunction with St Vincent's Research Valet Service for the provision of fee-based services
OCTOBER 2019
• ARC Training Centre for Cell and Tissue Engineering Technologies announced with CCRM Australia as partner organisation
JULY 2020
• First National Regenerative Medicine SuperPitch
APRIL 2020
• State Government of Victoria announces additional financial support for CCRM Australia
DECEMBER 2021
• Founding organisations renew financial commitment to CCRM Australia

CCRM AUSTRALIA HIGHLIGHTS

JULY 2020

First National Regenerative Medicine SuperPitch.

The SuperPitch program has been developed and specifically designed to provide a forum for a select group of venture funds to evaluate technologies curated for their specific interests in regenerative medicine. The program also provides selected applicants with expert feedback related to different stages of the commercialisation cycle and a potential source of alternative/non-government-based funding.

The inaugural National Regenerative Medicine SuperPitch was held virtually in July 2020. CCRM Australia curated four technologies that were presented to seven venture funds, three of whom were based in Australia. Companies and researchers showcased their technologies and ideas to translate and commercialise projects related to regenerative medicine.

The success of this and a further two SuperPitch events will see this Australian designed program extended to other CCRM hubs.

AUGUST–SEPTEMBER 2020

In collaboration with the BioMelbourne Network, CCRM Australia delivered a series of six stand-alone regenerative medicine focussed webinars, 'Breakthrough Technologies and Opportunities for Australia'.

With presentations from local and international speakers, the seminars delivered unique insights and knowledge on themes that drive business success locally and internationally, uniting the industry across research, translation and commercialisation, and progressing trade and export activities with key global markets.

Through virtual participation, attendees gained a deeper understanding of the local and global markets, the opportunities and challenges that exist for regenerative medicine businesses across the APAC region, and an insight into the successes and failures of those who have navigated the complex pathway to market before us.

OCTOBER 2020

CCRM Australia sponsors regenerative medicine session at Ausbiotech Conference for the fourth consecutive year.

Developed in conjunction with the Ausbiotech Regenerative Medicine Advisory Group, the session, 'Australian Developments in Regenerative Medicine', showcased advances, success stories, and novel treatments by Australian companies, focusing on sharing best practices and highlighting opportunities for further development.

The session featured speakers from the Australian Foundation for Diabetes Research, Regeneus, Cynata and Carina Biotech.

NOVEMBER 2020

CCRM Australia is commissioned by the Victorian Department of Jobs, Precincts and Regions (DJPR) to provide a report assessing regenerative medicine manufacturing options for Victoria

The report 'Advanced Therapies: A brief study of the current cell and gene therapy translation and manufacturing - Preparing Victoria for the future' makes a case for a translation facility to take advantage of a maturing regenerative market and engage the global market.

Recommendations include establishing a catalyst facility to drive more translation and commercialisation of regenerative medicine research and act as a landing pad for international biotechnology companies seeking to undertake preclinical and clinical development.

NOVEMBER 2020

Agreement to progress a CRC bid, the Solutions for Manufacturing Advanced Regenerative Therapies (SMART) Cooperative Research Centre (CRC)

In response to the growing partner network, CCRM Australia initiates a project to develop a proposal for 'the 'Solutions for Manufacturing Advanced Regenerative Therapies (SMART) Cooperative Research Centre (CRC). The SMART CRC will act as a collaborative, industry-driven catalyst that will overcome barriers to the Australian development and translation of these new frontier medicines from Australia's world-leading regenerative medicine research. The SMART CRC will also seek to introduce additional private sector funding into the program and encourage new company creation to provide cutting-edge therapies and platform technologies.

SMART CRC will encompass the broad array of research disciplines needed to tackle the significant challenges limiting the growth of the Australian regenerative therapeutics industry, including but not limited to developing therapies from stem cell research, genomics, materials science and engineering.

DECEMBER 2020

CCRM Australia founding members commit to financial and in-kind support to CCRM Australia.

Since its inception, CCRM Australia has relied on the support of its founding organisations, CSIRO, St Vincent's Melbourne, Monash University, the Australian Regenerative Medicine Institute and the CCRM.

Their recommitment underscores the value placed by these organisations to supporting Australia's regenerative medicine activities and the successful establishment of CCRM Australia.

APRIL 2021

CCRM facilitates the first of a series of roundtable discussions on specific items of interest.

The CCRM Australia – AbbVie Neuroscience Roundtable provide a forum for understanding (i) the current global strategic priorities in neuroscience research and development, (ii) the level of support for neuroscience research in Australia, and (iii) opportunities for neuroscience research, both globally and in Australia.

The resulting discussions allowed participants to better understand the current and future unmet needs in neurosciences research and opportunities for collaboration between universities and industries.

CCRM AUSTRALIA IN FOCUS

BUILDING BLOCKS OF UNIVERSITY-INDUSTRY COLLABORATION: REFLECTIONS FROM CCRM AUSTRALIA – ABBVIE NEUROSCIENCE ROUNDTABLE

By: Aashima Godara, Bobby Halim, Yamuneshwaran Ganapathy Kamal, Vaishnavi Deshamoni & Dr Chih Wei Teng

Increased rates of University-Industry Collaboration (UIC) was identified by the Australian Department of Education Skills and Employment (February 2021) as a critical mechanism to commercialise university research, thereby significantly boosting the country's economic development. The commercialisation of university research would often yield more significant productivity levels, leading to greater profits and more spinouts, thus contributing to business and job creation. One such model to facilitate discussions between universities and industries is a formal roundtable discussion, which typically involves key stakeholders representing strategic interests within the business and academic sectors, combined with executive support.

The recent CCRM Australia – AbbVie Neuroscience Roundtable is an example of the roundtable mentioned above involving AbbVie and Monash University research activities members, including the Australian Regenerative Medicine Institute, the Turner Institute of Brain and Mental Health and Monash Institute of Pharmaceutical Sciences. Special guests invited to the Neuroscience Symposium included BioCurate, an early-stage funding agency jointly formed by the University of Melbourne and Monash University, and Griffith Hack, a legal firm specialising in patents and trademarks.

The purpose of the CCRM Australia – AbbVie Neuroscience Roundtable was to provide a forum for understanding (i) the current global strategic priorities in neuroscience research and development, (ii) the level of support for neuroscience research in Australia, and (iii) opportunities for neuroscience research, both globally and in Australia. Thus, as the facilitator, CCRM Australia worked to achieve a better mutual understanding of each participant's capabilities and goals whilst ensuring that scientific innovation of neuroscience research is the prime force that both parties deliver the benefits to public health.

This blog seeks to summarise several key deliverables from the roundtable discussion that reflect the mutual benefits of UIC, especially when UIC is based on the desire to build a long-term engagement and sustainable partnerships.

"The commitment to neuroscience in AbbVie is broad, deep and going to be very long term and sustained, so from my perspective, the most important thing is to build a good working relationship, really have a kind of mutual trust, the clinical trials that we want to bring, and the data that I am getting." – **Dr Michael Gold, Vice President for CNS Development, AbbVie.**

Long-term associations with universities are advantageous for time-poor commercial companies by providing the much-needed basic level research knowledge. However, acquiring fundamental knowledge is a sacrifice that involves conceptualisation and operationalisation skills coupled with long preparation and analysis time. Arguably, all these are best found in academia. AbbVie highlighted this beneficial arrangement during the roundtable discussion, underscored by Dr Michael Gold's statement that he didn't want a transactional relationship where AbbVie simply pays in exchange for a service.

During the Roundtable, Prof Terence O'Brien and other representatives from Monash University shared their research and showcased the facilities they operate. In particular, the early Phase Neuroscience Clinical Trials Unit that Prof O'Brien manages at the Alfred Hospital drew significant interest from the AbbVie personnel. This is the second benefit of UIC, i.e., industry participants gain access to advanced technologies and cutting-edge academic knowledge, resulting in new opportunities to develop new products, enhance product quality, or even design solutions for production and service problems.

The long-term UIC brings with it the opportunity of potential staff and student exchange. These windows of opportunity provide an essential experience for both skilled and amateur personnel involved in these partnerships for their professional development, in addition to acting as a potential cornerstone in building sustainable mutually benefitting UIC. For example, an offer was made by **Dr Michael Gold, AbbVie,** during the CCRM-AbbVie Neuroscience roundtable, *"If you have a particular person that you think would be astounding in terms of learning, clinical trial, and sort of drug development from a business, from a pharmaceutical perspective that you want to then bring back into your shop for whatever reason. We should talk about whether that's an opportunity for somebody in your institution to come in and spend a year or two years with us, and then come back to you with a very different skill set".*

The Roundtable ended with both AbbVie and Monash University appreciating the need for an increase in dialogue between the organisations to flourish to their fullest and have expressed much interest in active communication to understand each other's expectations and objectives. Overall, the Roundtable discussion was crucial in allowing the participants to express their individual ideas, insights, and expectations from the collaboration and will enable them to extend mutual help and cooperation, consequently understanding the current and future unmet needs and opportunities in neurosciences research.

PS: Following the CCRM Australia – AbbVie Neuroscience Roundtable, Monash University extended an invitation to visit the Alfred Hospital's Phase 1 clinical trial site as a sign of proactive dialogue exchange.

ANTI-AGING THERAPIES MAY HAVE SETTLED THE ARGUMENT OF WHETHER AGING IS A DISEASE.

By: Dr Chih Wei Teng and Rupal Pichholiya

The introduction of novel medicines or medical procedures is never without controversy. History is littered with examples of humans attempting to 'play God or against nature'. Some modern cases include in vitro fertilisation, CRISPR Cas9 genetic manipulation and many others that sound more science fiction to the layperson, such as synthetic biology. However, perhaps the single oldest ambition of humanity in the research of medicine or human health is to cheat death from aging.

Anti-aging research from Professor David Sinclair of Harvard Medical School reopened the age-old (pardon the pun) debate on whether aging is a disease. Some of the best arguments are the opposing views of Bulterijs et al. (2015) and Gavrilova & Gavrilova (2017). The former made a compelling case of mislabelling a medical phenomenon because humanity lacked the understanding. When we better understand the phenomena due to improved diagnostics, instrumentation and knowledge, we are better at treating the effects of aging as if they are a disease. On the other hand, the later authors of Gavrilova & Gavrilova (2017) counterargued that age represents a maturation of age-related diseases as the body deteriorates, and therefore, it is not possible to establish a cause and effect relationship between age and the condition. Interestingly, the synthesis of the differing views is that advancements in diagnostics, instrumentation and knowledge can lead us to understand and identify the age-related effects or how the forces of natural selection increase the presentations of disease. Furthermore, knowing what inhibits or protects us from age-related diseases that govern our mortality allows us to target our therapies to extend or restore their functions, realising a fate that is free of age-related diseases that bring about an abrupt end to our natural lifespan.

If we view the statement from Professor Sinclair that 'aging is far more reversible than we thought' through the above lens, it might more be about extending the strength of a person's youth such that it continues to suppress the onslaught of age-related diseases. However, an internet search revealed results that describes Professor Sinclair's work as 'age reversal', 'getting younger, 'anti-ageing and 'fountain of youth'. This begs the question, are these discoveries medicine, nutraceutical or cosmeceutical?

While we should not seek to suppress innovation in health and medicine, there are concerns about nutraceuticals and cosmeceuticals having less scrutiny or being evaluated with less rigour than traditional drugs. Some proponents view a designation as a nutraceutical or a cosmeceutical as a fast track to market or as a means to exploring new business models and shunning the conventional chemists or pharmacies in favour of online selling or multi-level marketing arrangements. Chemists and pharmacists play an essential role at the grassroots level to safeguard against the abuse of medicines. More vigilance and scrutiny are needed as novel therapies entering the nutraceuticals and cosmeceuticals space are utilising processes and tools commonly found in advanced therapeutics medicinal products (ATMPs). A good example is Professor Sinclair's cell reprogramming experiments, which showed axon regeneration in glaucoma mouse models when injected with vectors loaded with genes from a cocktail of reprogramming factors.

The risk lies not so much with the claims but in the inherent processes within the production and formulation of ATMPs and the ability to sell ATMPs through non-traditional means that are difficult to trace and recall. To that end, it might be pertinent that we begin to recognise aging as a disease, not because of philosophical reasons but simply that anti-aging has evolved away from merely treating 'skin deep' conditions to actual therapies that treat age-related diseases such as Parkinson's or prevent cancer.

References

Bulterijs S, Hull RS, Björk VC, Roy AG. It is time to classify biological aging as a disease. Front Genet. 2015 Jun 18;6:205.

Gavrilov LA, Gavrilova NS. Is aging a disease? Biodemographers' point of view. Adv Gerontol. 2017;30(6):841-842.

Lu, Y., Brommer, B., Tian, X., Krishnan, A., Meer, M., Wang, C., Vera, D. L., Zeng, Q., Yu, D., Bonkowski, M. S., Yang, J. H., Zhou, S., Hoffmann, E. M., Karg, M. M., Schultz, M. B., Kane, A. E., Davidsohn, N., Korobkina, E., Chwalek, K., Rajman, L. A., … Sinclair, D. A. (2020). Reprogramming to recover youthful epigenetic information and restore vision. *Nature,* 588(7836), 124–129.

SUCCESS IN INTERNATIONALISATION IS NOT ONLY DETERMINED BY TECHNICAL PROWESS.

By: Mahmuda Khatun. Edited by Dr Chih Wei Teng

The exponential growth of regenerative medicine (RM) has attracted many venture businesses and companies seeking to discover newer therapeutic interventions that mitigate human suffering; creating employment opportunities and increasing workers productivity (4). This growth is also fuelled by high levels of private and government investment as evidenced by an increase in global financing to $9.8 billion (1) and the Australian government investment of AU$5.6m in RM skills development programs in May 2018 (2).

Of the many challenges that await the bio entrepreneur, including safety, regulatory science, and standardisation, manufacturing, cell viability, talent & suitable workforce shortages (5), the internationalisation process stands apart. This is because internationalisation is a challenge that is rarely discussed in detail except for market size of different jurisdictions and their respective regulatory frameworks. In other words, internationalisation is one challenge for which most biotechs are the least prepared.

It is essential to understand that developing viable products and having a clear business model or strategy are not the only pre-qualifications for entering the international market. Developing a qualified workforce with appropriate management skills, including cross-cultural communication, to implement the business plan, are some essential considerations before entering into the international market. The purpose of this blog is to enlighten the importance of both technical and soft skills for biotech start-ups, which need to be balanced when shaping the workforce before entering into the global regenerative marketplace.

A biotech's strategy will dictate the company structure and functions needed for internationalisation. Whether the structure will include scientists from research, technicians from manufacturing facilities, or personnel from sales and marketing having the right mix is critical for achieving the company's goal. To be more specific, if the biotech plans to manufacture overseas with a contract development/manufacturing organisation (CDMO), then the researchers' team with a broad range of skills, ranging from basic technicians to PhD-level personnel are required to interface and project manage. If the biotech chooses to be vertically integrated with its own manufacturing facilities, personnel with strong skills in process engineering, quality control, production technology, and project management are essential. Also, personnel should have good knowledge of GMP (or Good Gene, Cellular, and Tissue-based Products Manufacturing Practice [GCTP]) as well as manufacturing experience and medical and pharmaceutical knowledge (6).

When it comes to sales, marketing and customer services for a global market, having a talented and diverse team with experience in consultative selling, good communication skills, and effective customer relationship management skills are essential criteria. This critical element was recently illustrated in a webinar co-organised by the BioMelbourne Network and CCRM Australia. During this webinar, "International Market Entry - Some Shared Insights", guest speaker **Mr Paul Brennan, Managing Director of leading Australian regenerative medicine company Polynovo Limited,** shared his journey in introducing, developing and promoting the product named **'Novo Sorb BTM'**, a biodegradable temporising matrix, used for regeneration of lost dermis via surgical application. This unique product is currently marketed in 12 countries, including Australia, New Zealand, USA, Singapore, Malaysia, Germany, Austria, Switzerland, Saudi Arabia, Israel, South Africa, and India. Polynovo has plans to expand the product's availability to Taiwan, South Korea, Finland, Norway, Benelux, France, and Kuwait throughout 2021. When articulating his strategy in internationalising into the global market, Paul specifically touched on developing a team that possess several characteristic traits such as passion, commitment, nimbleness, openness, focus, up to date, respect and empathy. The need to mitigate cultural, language, traditional barriers are significant, and even more now with Polynovo's product being marketed in so many countries.

Internationalisation necessitates working in a diverse workplace with people having different mindsets. Having a clear understanding of the behaviour patterns of high context societies (establishing relationships and trust) is paramount. Low-context societies (business-like and formal) more effortlessly adapt communication styles and build the right relationships with international colleagues. Moreover, the ability to understand, learn and appreciate cultural differences will assist in improving cross-cultural communication, teamwork, capabilities, flexibilities, responsiveness, trust, and royalty among local suppliers as well as partners. In any business, disputes will arise, leading to negotiation. A team with the right 'soft skills' including interpersonal influence, adaptive thinking, emotional intelligence and resilience that is better able to deescalate situations and work towards a win-win situation effectively is an asset.

To sum up, human resources are the primary building blocks of a company (7). Not only focusing on technical skills but also balancing the workforce with soft skills is one of the primary considerations for bio entrepreneurs. The impact of this consideration was evidenced during the Covid-19 pandemic. The lack of personal interaction and restricted travel made it a challenging time for bio entrepreneurs. Many companies turned towards IT-based solutions, including video conferencing or more sophisticated customer relationship management systems to provide continuity in their international business. However, IT solutions are merely multi-lateral communication vehicles, rapid data analysis tool with dashboards, tailored to each business function. Without an effective workforce that has effective soft skills and a good cross-cultural understanding, biotech companies will not be able to build new relationships along with maintaining and growing existing relationships with the customers.

References:

1. Medicine AfR. Advancing Gene, Cell, & Tissue-Based Therapies ARM Annual Report & Sector Year in Review 2019 2020, Mar 20th [Available from: https://www.regenhealthsolutions.info/wp-content/uploads/2020/03/2019-ARM-Annual-Report-Sector-Year-in-Review.pdf].
2. Connect M. Regenerative Medicine Opportunities For Australia October 2018 20q8, Oct 10th [Available from: https://www.mtpconnect.org.au/images/MTPConnect%20Regenerative%20Medicine%20Report.pdf].
3. Markets Ma. Regenerative Medicine Market by Product (Cell Therapies (Autologous, Allogenic), Stemcell Therapy, Tissue-engineering, Gene Therapy), Application (Wound Care, Musculoskeletal, Oncology, Dental, Ocular), Geography - Global Forecast to 2025 2020, September [Available from: https://www.marketsandmarkets.com/Market-Reports/regenerative-medicine-market-65442579.html].
4. Hussain A, Rivers PA. The economic value of investing in regenerative medicine. Journal of Health Care Finance. 2009;36(2):45-54.
5. Polak DJ. Regenerative medicine. Opportunities and challenges: a brief overview. Journal of The Royal Society Interface. 2010;7(suppl_6):S777-S781.
6. Nishigaki F, Ezoe S, Kitajima H, Hata K. Human resource development contributes to the creation of outstanding regenerative medicine products. Regenerative Therapy. 2017;7:17-23.
7. Finegold D, Frenkel S. Managing people where people really matter: the management of human resources in biotech companies. The International Journal of Human Resource Management. 2006;17(1):1-24.

REAL WORLD EVIDENCE: WHY IS IT STILL INFERIOR IN EVIDENCE HIERARCHIES?

By: Aashima Godara and Dr Chih Wei Teng

Regenerative medicine and other advanced therapeutic medicinal products have revolutionised modern medicine, offering transformational, long-lasting and potentially curative outcomes for many disorders [1]. Like other medicines that come before them, regenerative medicine has to go through randomised controlled trials (RCTs) to gather evidence on safety and efficacy. However, concerns begin when RCTs provide clinical data derived from a small subset of the patient population and a limited number of predefined consultations. To more properly know the utility of a medicine (i.e. costs vs benefits), there is a need to capture the total magnitude of potential gains and effects over a long period. Autologous regenerative medicines, other forms of personalised therapies and therapies for rare diseases tend to recruit a lower number of recruitments for RCTs. As a result, there are concerns for unknown effects in the broader population, especially those with different medical conditions [1].

Leveraging real-world evidence (RWE) to complement and strengthen the RCT data will provide a more holistic picture post regulatory approval. Electronic health records (EHRs), medical claims, disease registries, etc., can be used to complement clinical data for pharmacovigilance and renegotiating health technology assessments (HTA) [2,3]. These can provide invaluable guidance inadequately captured by clinical trials. This includes changes in patients' behaviour and mental state. RWE can become crucial following regulatory approval as it can synthesise authentic and significant data throughout the product life cycle to help boost the uptake of regenerative and advanced therapies [1]. Moreover, RWE can be used to renegotiate reimbursement/subsidised pricing of advanced therapies [1].

However, it is still not considered best practice to integrate RWE into regulatory assessments. The lack of consistency and harmonisation within countries regarding RWE has been commonly cited as a hurdle [4]. In a report where HTA agencies in major European jurisdictions were assessed on the policies for the use of real-world data (RWD), the conclusions showed disapproval [1], distrust in using RWD amongst the stakeholders and; [2] the policies surrounding the use of RWE varied across the HTA agencies [4]. A survey conducted in Asian countries drew similar conclusions and further suggested a lack of infrastructure required to gather the data and the skills needed to analyse the data [5].

Recognising that every jurisdiction has its issues surrounding RWE, whether it be lack of infrastructure or trust, etc., some initiatives are proposed, and others implemented to boost the use of RWE. For instance, using a single identification number for patient tracking in different RWE sources such as EHRs and medical claims [6]. This approach could promote consistency in RWE and easier use of the evidence. Another step could be to harmonise the RWE sources, for e.g. a single generally accepted format amongst the different jurisdictions could capture more insightful and interoperable information [6]. Resolving the issues of consistency and harmonisation in RWE could lead to more recognition, trust, and use of such evidence. The issue of infrastructure and skills to analyse and collect RWE has been raised as an emerging topic in countries including the United States [7]. A reliable infrastructure for pragmatic research could lower costs and provide easy and quick access to cohort studies. Introducing the necessary skillset and framework could bring the RWE closer to a more central role in medical product's regulatory and safety monitoring activities [7].

Hesitancy about regenerative medicine and other advanced therapies could be eliminated by providing more comprehensive evidence. A comprehensive RWE register is needed for advanced therapies, both present-day and in the future. This register would support the clinical data, not just for regulatory approval but to help to properly evaluate and thus realise the benefits of these modern treatments.

References:

1. Mihos M, Spinner D, Ringo M & Faulkner E. Leveraging Real-World Evidence for Regenerative Medicine and Advanced Therapy Success Beyond the Regulator. Evidera.
2. Booth A. How Health-Related Social Media Can Complement Traditional Real-World Evidence Approaches to Offer Unique Patient Insights. Evidera.
3. McDonald L, Malcolm B, Ramagopalan S & Syrad H. Real-world data and the patient perspective: the PROmise of social media? BMC Medicine.
4. Makady A, Ham R, Boer A, Hillege H, Klungel O & Goettsch W. Policies for Use of Real-World Data in Health Technology Assessment (HTA): A Comparative Study of Six HTA Agencies. Elsevier.
5. Lou J, KC S, Toh KY, Dabak S, Adler A, Ahn J, et al. Real-world data for health technology assessment for reimbursement decisions in Asia: current landscape and a way forward. International Journal of Technology Assessment in Health Care.
6. FRAMEWORK FOR FDA'S REAL-WORLD EVIDENCE PROGRAM [Internet]. U.S. FOOD & DRUG ADMINISTRATION; 2018 [cited 7 June 2021]. Available from: https://www.fda.gov/media/120060/download
7. Cavlan O, Chilukuri S, Evers M, Westra A. Real-world evidence: From activity to impact in healthcare decision making [Internet]. McKinsey & Company. 2018 [cited 7 June 2021]. Available from: https://www.mckinsey.com/industries/pharmaceuticals-and-medical-products/our-insights/real-world-evidence-from-activity-to-impact-in-healthcare-decision-making#

NATIONAL REGENERATIVE MEDICINE SUPERPITCH III - OBSERVATIONS

By: Rupal Pichholiya, Sarmad Sonde, Vaishnavi Deshamoni and Wilma Lopes

INTRODUCTION

Having an opportunity to observe presentations delivered by companies seeking to launch innovative products into the market has undoubtedly enlightened us as CCRM Australia interns to the do's and don'ts of pitching to investors. The SuperPitch series have been designed to provide a forum for a select group of venture funds to evaluate technologies curated for their specific interests in regenerative medicine. This event also provides selected applicants with expert feedback related to different commercialisation cycle stages and a potential source of alternative/non-government-based funding. During the SuperPitch event, we also noted the questions asked and discussions between presenters and investors gave us an insight into what investors are concerned with based on the commercialisation stage and who is driving the innovation, i.e. clinician- or sponsor-led. Based on this experience and knowledge gained from our Master's course in biotechnology and entrepreneurship units, we would like to synthesise our understanding and present a number of key factors that will contribute to the success of a pitch. We hope the following factors should serve as a guide to develop an impressive pitch that targets the potential investors, intrigue them about the product and engages their interest.

1. Value proposition:

An excellent start to any pitch is to quickly establish a value proposition that summarises the product and draws the audience attention towards differentiating factors that separate it from the competition and promised benefits. We were particularly impressed by value propositions that go beyond 'why' a customer would be convinced in favour of the offer or 'what' is the better value when compared to competitors, to include how may the innovation be scaled or deployed beyond a single medical institute or geography to improve public health or inspire when patient experiences are woven into the storytelling? One such example related to shared patient experiences, which connected with the audience and shared a story about how the solution had a personal connection with the patient. This has set the presentation apart from others, evoked an emotional response and sparked interest in further conversation.

2. The issue:

A crucial factor that establishes the value of a product is the problem that it aims to solve. When a company can articulate the problem, not just financially but also the quality of life burden, it captures the audience's attention. It also characterises the problem not as a statistic but as a person struggling. This is beneficial because it creates a base for the solution offered, makes it more relatable, and brings a sense of adventure and triumph. The audience can then understand both market potential and its valuable contribution to a better quality of life.

3. The solution:

The solution presents an innovative approach to solving the issue and is one of the most important sections of the presentation. The presenter describes the solution details and how it differs from the previous or existing solutions, preferably by explaining the mechanism of action. In addition, one of the best strategies is to emotionally connect with the viewers by building a captivating product storyline that aims to resolve the pain point experienced by patients.

4. Market competition:

Every business venture must have an insight into the current competitive landscape and should present how they either fit into it or stand out. Explanation about the present-day market competition is one of the inevitable parts while pitching to the investors. It is beneficial to know the existing treatments (if any) and awareness of their positive clinical outcomes and adverse effects. A lack of explanation of the current market landscape might be taken negatively if it were suspected to artificially enhance the value of the product or a lack of awareness about the competition on the presenter's part. One of the presenters provided a list of their competitor companies along with their valuations, impressing the audience with their due diligence.

5. Product roadmap:

A product roadmap can be a crucial part of a pitch as it defines short and long-term priority areas. In addition, investors are impressed if an organized timeline is used to explain the breakdown of costs associated with the business model from the developmental stages to the market, supporting IP strategies and future goals.

6. Use of investment/funds:

It is essential to understand how much money is required, but knowing how that money needs to be spent is trickier. This point captures the viewers' attention when the narrator presents the milestones to be achieved at each funding stage, the go-to-market plan, and the expected growth projections. Investors are always concerned about how their investment would be utilized to benefit the company and evaluate if the presenter has missed any critical steps in the commercialisation journey and whether the budget is realistic. Also essential is the investment timeline that reflects fundraising plans, increasing the company's valuation and, ideally, some potential exit strategies.

CONCLUSION

The SuperPitch is a platform where one witnessed the storytelling of how ideas are transformed into technology and subsequently technology into commercialisation. These factors were based on our opportunity to observe SuperPitch presentations that embodied different styles, reactions from investors, lines of questioning to the presenters and reflections between the investors. The purpose is to share what we feel would provide a guide to develop a pitch that would capture and interest the audience, and especially the investors.

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Dr Jack Lambshead (till December 2020)
Dr Wade Kruger (from January 2021)

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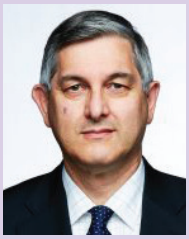

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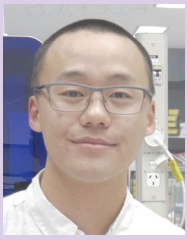

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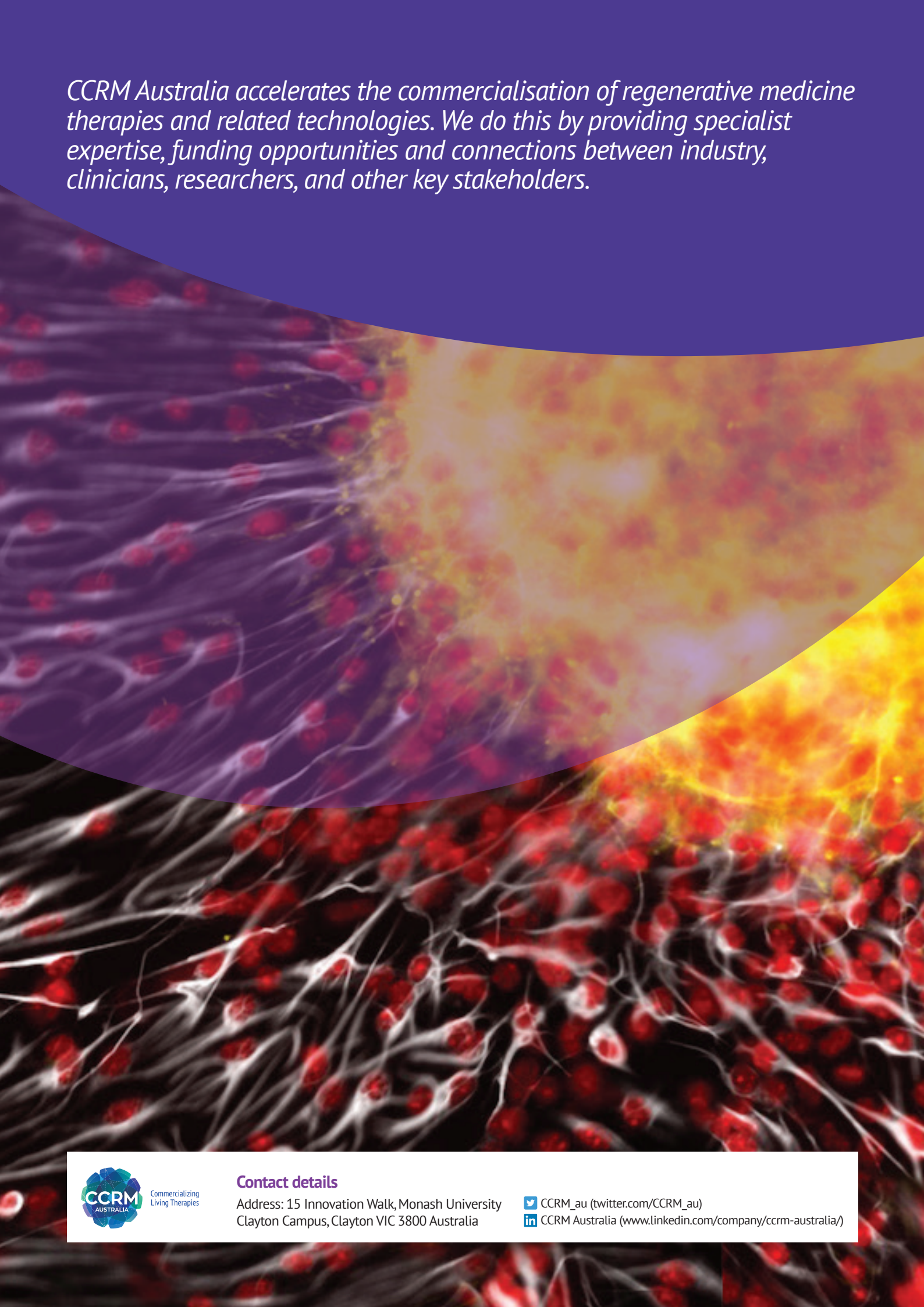
CCRM Australia accelerates the commercialisation of regenerative medicine therapies and related technologies. We do this by providing specialist expertise, funding opportunities and connections between industry, clinicians, researchers, and other key stakeholders.



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