Biotech Daily

Thursday December 19, 2024

Daily news on ASX-listed biotechnology companies

*** ASX, BIOTECH DOWN: MESOBLAST UP 54%; RESONANCE DOWN 12%**

*** 2024 – THE YEAR IN REVIEW**

*** FDA APPROVES MESOBLAST RYONCIL FOR GvHD**

*** PETER MACCALLUM 'CRISPR SILENCES CANCER-CAUSING RNA'**

*** ORTHOCELL FILES FDA REMPLIR 510(k) APPLICATION**

*** CERTA, OCCURX MERGE FOR FIBROSIS TREATMENTS**

*** STARPHARMA 'POSITIVE' FDA FEEDBACK FOR DEP-SN38 PATHWAY**

*** FDA TO INSPECT EBR MANUFACTURING IN JANUARY**

*** BLINKLAB: FDA MAJOR TRIAL FOR AUTISM TEST**

*** MAYNE: 'COURT APPROVES $38m CLASS ACTION SETTLEMENT'**

*** LTR, MEN'S HEALTH DOWNUNDER PARTNER FOR SPONTAN**

*** FIREBRICK EXTENDS KEITH SHORTALL FOR SINGAPORE NASODINE SALES**

*** LITTLE GREEN WELCOMES WA MARIJUANA REGULATION CHANGES**

*** IMUGENE RECEIVES $11m FEDERAL R&D TAX INCENTIVE**

*** RADIOPHARM PLEADS 'BEST PRACTICE' TO ASX AWARE QUERY**

*** MARK LAMBERT, BVF REDUCE TO 7.3% OF ACTINOGEN**

MARKET REPORT

The Australian stock market fell 1.7 percent on Thursday December 19, 2024, with the ASX200 down 141.2 points to 8,168.2 points. Nine of the Biotech Daily Top 40 companies were up, 25 fell, five traded unchanged and one was untraded. All four Big Caps fell.

Mesoblast was the best (see below), up as much as 56.1 percent to $3.09 before closing up $1.07 or 54.0 percent at $3.05, with 57.8 million shares traded. EBR climbed 14.5 percent; Cynata was up 11.4 percent; Micro-X rose 9.1 percent; Aroa, Starpharma and Syntara were up three percent or more; with 4D and Opthea up by more than one percent.

Resonance led the falls, down 0.7 cents or 11.9 percent to 5.2 cents, with 716,641 shares traded. Genetic Signatures lost 6.6 percent; Atomo, Avita, Cyclopharm, Dimerix, Imugene and Polynovo fell five percent or more; Curvebeam, Immutep and Pro Medicus were down more than four percent; Actinogen, Clarity, Medadvisor and Proteomics lost more than three percent; Amplia, Medical Developments, Neuren, Paradigm, Prescient, Resmed and SDI shed more than two percent; Cochlear, CSL and Nanosonics were down one percent or more; with Clinuvel, Emvision, Orthocell and Telix down less than one percent.

2024 – THE YEAR IN REVIEW

Despite all the naysayers, 2024 was brilliant for most biotechs, with a record year for raising capital (see below).

The Biotech Daily Top 40 Index (BDI-40) hit record highs throughout the year, more companies than ever posted revenue and some even had very unbiotechy profits.

A year of head down and hard work, with lots of financial reports and trial data. In fact, far too much data, with one or two companies letting us know every morsel of development. We think Nyrada posted the most pre-clinical data, but Immutep and Imugene confused readers with too much trial data. The announcements really should be the start of the trial and the results. A couple of paragraphs at the half-way mark and another couple when dosing has been completed. The Marrakesh Express also stops at every cow.

Volpara was acquired by Seoul's Lunit Inc for $295.7 million, we lost Rhinomed from the ASX but the company continues. Medlab Clinical continues in suspension, we're not sure what's happening at Epsilon, and Auscann has been removed from the official list. Medical Grade Cannabis (MGC) changed its name to Argent and migrated to the London Stock Exchange at a market capitalization of $46 million, spiking above $300 million and is leaving the LSE currently valued at $12 million.

Creso Pharma changed its name to Melodiol last year and disappeared altogether. Allegra was bought by shareholder and director Dr Nicholas Hartnell.

Anteris has listed on the Nasdaq. It's share price is an impressive $9.00 but that follows a 10-to-one consolidation, followed by a 100-to-one consolidation, making the company worth 0.9 cents a share compared to the 17.0 cents at which it was trading in October 2014 when the then Admedus (previously Allied Medical and prior to that Biomd) appointed chair John Seaberg and managing-director Wayne Paterson as directors.

Hexima still exists, but terminated its deal with Real Thing Entertainment Pty Ltd. Perhaps production assistance by Ian 'Molly' Meldrum was required. IDT was going to be taken over by North Melbourne psychedelics company Myndbio, but that proved illusory.

Exopharm became Tryptamine. Regeneus became Cambium, retained its chief executive officer and did not much, at all. Cann Global was delisted by the ASX and some were not unhappy with the decision.

Percheron (formerly Antisense) had ATL1102 renamed as "avicursen" and Starpharma renamed its dendrimer enhanced product (DEP)-irinotecan as DEP-SN38. Pharmaxis became Syntara (not to be confused with either Cynata or Marc Sinatra).

Vitura sold nicotine vapes claiming they were 'smoking cessation products'.

Too many Vales

The sad news was the large number of biotech-related deaths this year.

We deeply mourn several great losses, not least of which was our very own marijuana and psychedelics correspondent (Piotr) Peter Olszewski – the legendary JJ McRoach.

But the year started with the demise of major biotech investor Lang Walker and continued with luminaries including the doyen of Australian biotechnology, Leon Serry, along with Vectus chief executive officer Dr Karen Duggan, Qbiotics chair Dr Susan Foden and long-standing colleague and former Peptech and Arana chief executive officer Dr John Chiplin.

We also lost Nobel Laureate Dr (John) Robin Warren, who worked with Prof Barry Marshall in the discovery of the Helicobacter pylori bacterium.

A.I. & A.S.

Artificial intelligence – previously known as computer software – remained the biotech buzzword of the year with more companies claiming to be 'A.I.-this' or 'A.I.-that', having learnt how to program their computers.

But in the real world, we quickly discovered A.I.'s sibling A.S. through myriad programs. It's called Artificial Stupidity - also known as GIGO: garbage in, garbage out.

February

Proteomics expanded from diabetic kidney disease detection to endometriosis and oesophageal cancer, Oncosil expanded its pancreatic cancer treatment to Türkiye and more German hospitals, the Federal and Victoria governments told us they were pouring millions of dollars into all sorts of medical research and commercialization, CSL clearly said the CSL112 cardiac trial missed its primary endpoint and dropped the program, but the next day announced record half year revenue and profit.

The US Food and Drug Administration refused Starpharma's appeal to approve Vivagel BV for bacterial vaginosis maintaining the need for "additional clinical efficacy data" - in other words: another trial.

A number of companies took Endpoints Capital and Radium RDTI loans along with a raft of draw-down equity facilities. The best was Inhalerx with a market capitalization of $6 million claiming it had an investment of $30 million, which turned out to be a draw-down equity facility.

More mice showed that drugs worked for all sorts of cancers, including Race's bisantrene for acute myeloid leukaemia and Invion's INV043 curing 80 percent of mouse anal cancers. Mice also had improvements in heart conditions, vasculitis, sepsis, melanoma, psoriatic arthritis and Alzheimer's disease.

In April, Aegros said it hoped to raise $100 million to become Australia's biggest blood fractionator (don't mention CSL) and last month Aegros executive chair Dr Hari Nair told Biotech Daily the company hoped to "complete a raise prior to Christmas".

Dr Nair also provided "a flat denial" to a question about Dr Esra Ogru working with Aegros.

There were persistent rumors that the convicted thief and former Phosphagenics chief executive officer Dr Ogru was working with some unnamed biotechs, but no one was boasting of her acquisition. And none were speaking “on the record”.

Blinklab jumped 50 percent on its initial public offer, closing up 32.5 percent at 26.5 cents for its autism and neurological test.

Neurotech marijuana NTI164 met endpoints for autism and Rett syndrome, claiming the latter’s 14-patient trial “compares favorably” with Neuren’s 187-patient trial.

The Federal Court of Australia handed former Creso Pharma chair Adam Blumenthal a fine of $850,000 and banned him from managing corporations for five years, following an Australian Securities and Investments Commission and Australian Federal Police investigation of his dealings as the chair of Everblu Capital and “alleging market rigging and breaches of his duties as a director” of Everblu and Creso.

Pharmaust replaced chief executive officer Dr Michael Thurn in April and after a complete changing of the guard, losing long-time chair Dr Roger Aston along with directors Rob Bishop, Dr Tom Duthy and a little later Sam Wright, Dr Thurn returned in May to rename the company Neurizon, turning Elanco’s ‘monepantel for sheep round worm’ to the much snappier ‘NUZ-001 for amyotrophic lateral sclerosis (ALS)’.

Ausbiotech appointed Rebekah Cassidy as its new chief executive officer and Bio-Melbourne appointed Ausbiotech’s Karen Parr as its chief executive officer.

Nanosonics finally announced the FDA filing for its Coris endoscope cleaning system. In 2022, the company said it expected non-US approvals from the end of 2023 but gave no timeline for US approval.

Cochlear completed its Oticon acquisition. Pro Medicus wrote a further $45 million in five new Visage deals.

July

The first day of the new financial year began with the BDI-40 at an 18-year record high - up 847 percent over 18 years, compared to the ASX200 up 53 percent in the same time.

Brandon Capital raised $270 million for its Biotech Investment Fund VI. The next day Immutep said it had raised $90 million, with $10 million more to go. Radiopharm raised $70 million. So much for the lack of venture capital.

Epsilon (previously The Hydroponics Company) founder Alan Beasley won the fight for the company but we’re not sure what was meant to happen once he did.

Telix decided to waste a lot of time and effort listing on the Nasdaq and raising $300 million, but pulled the plug eight days later (we could have told them, but no one listens).

The FDA approved Botanix Sofdra for underarm sweating, the TGA approved Control Bionics’ Drove wheelchair module and Imricor conducted its first atrial flutter ablation.

Tryptamine dosed its first psilocybin patient, Patrys said PAT-DX1 testing was late, again, Mesoblast re-filed its application to the FDA for Ryoncil for paediatric graft versus host disease, for the third time, with the FDA surprising everyone with today's approval.

In the US, Kazia had some good news from its Paxalisib brain cancer trial and jumped 248 percent to 98.8 cents. It's market capitalization reached $35 million on December 1, 2024, a long way from the $207 million in 2021 two years before it went to the Nasdaq for the better valuations of the more informed US punters.

Polynovo was the first to post record revenue for the year to June 30 "up 58 percent to $105 million" and was far from the last. Telix raised a record $650 million through convertible notes – possibly the biggest in Australian biotech outside the Big Caps. Medadvisor, Micro-X and Cardiex posted record receipts from customers.

Bivacor implanted its first artificial heart and although much of the work was done in the US, we still claim the Oneventures-backed company as one of ours.

In August, for reasons we can't fathom, Israel's Redhill Biopharma, which bought Sydney's Giaconda in 2010, leapt 98 percent on six-year-old data. We don't begrudge them the uplift, but the data on Myoconda (RHB-104) for Crohn's disease was not new.

There is a general rule in Australian biotech: When a company releases good news, the share price goes down. When a company announces bad news, the share price goes down. When a company announces no news, the share price goes down. Occasionally, for no discernible reason, whatsoever, the share price goes up.

All four Big Caps - Cochlear, CSL, Pro Medicus and Resmed - posted revenue and profits up, probably all to record levels. Telix declared an H1 maiden profit, following the full year maiden profit for 2023. Argent (MGC) said it would delist from the ASX, but by November had changed its mind and decided to stay on the ASX but delist from the LSE, while maintaining a presence on the US over-the-counter market.

And fewer companies than ever before still failed to comprehend the memo that their RDTI was not revenue. The message is getting through to most, but there are some that think their punters are truly stupid and won't read the P&L statement.

EBR filed its final module to the US Food and Drug Administration for its Wise heart pacing device, with a 'yay' hoped for soon in the new year.

Then something strange happened. Biotech Daily received an email from the Federal Takeovers Panel saying the matter of Tissue Therapies had been referred. It went nowhere - in itself - but founder and chief executive officer Tony Charara was the last person standing after the applicants to the Takeovers Panel failed and chair Jack Lowenstein and directors Brian Gray and Dr Michael Silberberg left the building.

In August, Bioxyne said it had an up-to $28 million deal with an unnamed company to supply marijuana gummies, later telling the ASX it had no idea why its share price climbed 143 percent, other than the $28 million marijuana gummies deal.

By mid-September, the ASX suspended Bioxyne for not providing specific information about the $28 million marijuana gummies deal and a few days later, Bioxyne fessed-up the customer was Melbourne and Berlin's Montu Group Pty Ltd.

Woke added LSD to its mushroom psylocibin pipeline, making it one of the most trippy workplaces to be. And Patrys had more delays with PAT-DX1 manufacturing.

October began with two august bodies claiming that it was hard to raise funds for Australian biotech. Unfortunately for them, the facts were opposite. This year has been a record year for capital raisings (see chart and commentary below).

We are aware that some companies have struggled to raise funds and some have technologies we think deserve funding. But the truth is that of all the companies having difficulty raising money, many have drugs, devices, diagnostics or software that don't appear to be very interesting, or benefit human health or are likely to make a buck (see the 2024 Kamikaze Award below).

Patrys gave up on PAT-DX1, refocussing on the full antibody PAT-DX3. Patrys chief executive officer Dr James Campbell said that both were anti-cancer and inflammatory molecules, but DX1 was smaller and "penetrated the blood-brain-barrier a bit better".

Then came the onslaught of pre-revenue companies thinking it a good idea to give directors and c-level staff options, shares, performance rights and restricted shares, before giving investors dividends.

We have tried to point out how foolhardy this is for nearly two decades, but not many are listening. Some of the best companies don't do it all. Some of the worst do it all the time. The most amusing are those who stick their noses in the trough when they already have a remuneration report first strike, but simply don't "get it".

November gave some interesting results.

Monash University did a lot of good work through the year, as did other universities and research institutes.

One of the best headlines we had the opportunity to write was that 'Psilocybin May Aid Depression, In Rats'. We don't know how one would begin to study that, but the next time we see a depressed rat, we'll send it mushroom foraging in the Dandenong Ranges or on Mt Macedon or as some sources we heard in a pub and would never recognise if we saw them again, officer, say: try Malvern Valley (Gardiner's Creek) or even along the Yarra.

The FDA approved Echo IQ's Echosolv for aortic stenosis, Prof Andrew Wilkes and Dr Chris Burns won the well-deserved $250,000 Prime Minister's Prize for Innovation for inventing and commercializing Ojjara, previously known as momelotinib or CYT387, for myelofibrosis; and Ausbiotech ran its "biggest" conference and bio-invest summit, with Bivacor chief executive officer Dr Daniel Timms giving the Prof Nancy Millis Oration.

November kicked-off with the biggest change to the Biotech Daily Indices: Pro Medicus was promoted to the Big Caps, having overtaken Cochlear's market capitalization.

Always Remember the 5th of November

That used to be the slogan for Guy Fawkes Night – also known as Bonfire or Cracker Night. But this year it took on a whole new meaning as President Donald John Trump won a second US election and announced his new Health Czar as Robert Francis Kennedy Jr.

With a month to go to Inauguration Day, we have no idea whether Junior will be libertarian and let everything through the FDA, or continue his anti-science agenda, banning vaccines, removing fluoride from water and preventing anything passing the FDA. According to the Washington Post, the new head of the FDA is Dr Marty Makary, a Johns Hopkins surgeon, with vaccine sceptic Dave Weldon running the Centres for Disease Control and Prevention. The Post said Dr Janette Nesheiwat was a medical doctor chosen to be the next Surgeon General but was better known as a Fox News commentator.

And all report to Junior.

Back home, Neuren received $76 million for its share of Acadia's sale of the Daybue (trofinetide) rare paediatric disease priority review voucher, which was separate from a $76 million Daybue milestone. Bionomics soared 215 percent from 19 US cents to 60.5 US cents on news that it would be paid a $1 million milestone by Adelaide's Carina Biotech, which just happens to be helmed by Bionomics former chief executive officer Dr Deborah Rathjen. Yesterday, the share price had fallen back to 26.2 US cents.

And then the opposition to directors' stock and remuneration reports began in earnest. The strongest vote against a remuneration report was Island with a 92 percent second strike and 98 percent board spill vote, but it wasn't on its own. Biotron copped a 55 percent first strike and lost two directors; Atomo had a 44 percent second strike; Rhythm earned a 42 percent first strike; Imugene and Percheron both had 35 percent first strikes (with Percheron withdrawing two resolutions) and 4D copped a 32 percent first strike.

On November 20, Genetic Technologies appointed administrators. A long time coming and just eight days short of the 10th anniversary of founder Dr Mervyn Jacobson being sentenced to 12 months porridge (see below).

And before you could say "Summer holidays" it was December and the BDI-40 was at its 11th record high for the year. The only month it slipped was August.

Micro-X had a funny start to Summer having to tell the ASX that a US Government website had announced a $12.75 million contract before sealing the deal; and the following day, formally announcing the contract.

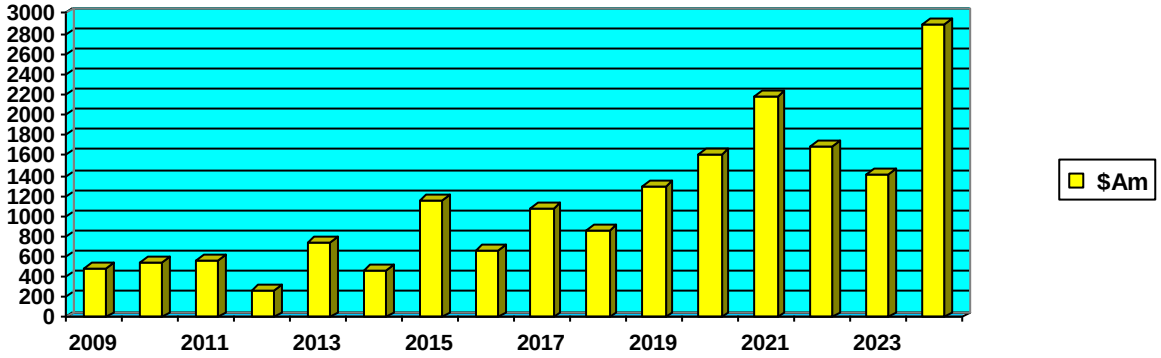
Nobel laureate Prof Gus Nossal donated a "big wadge" of cash to The Walter and Eliza Hall Institute providing a perpetual professorship in his name. Professors are paid around $180,000 a year, so a perpetual donation would be at least $2 million. We like Gus!

The Monash University Moderna mRNA vaccine factory was opened by the Federal and Victoria Governments. Anteris said it would redomicile in the US, pending a successful initial public offer, which it did; and Pro Medicus founders Dr Sam Hupert and Anthony Hall sold one million PME shares each at $256.73 a share. Why didn't this writer buy shares at 70 cents each when he had the chance?

Visioneering asked the ASX to delist, having failed to commercialize its contact lenses and one biotech executive claimed companies were leaving the ASX and not listing due to excessive costs and corporate governance burdens. One problem facing the ASX is that in the absence of a proper regulator (the Australian Securities and Investments Commission is as useless as mammary glands on a male bovine), the ASX is both the de-facto regulator, and a fuddy-duddy gentleman stockbrokers' club. The year ended with Mesoblast climbing to a $2 billion market capitalization before the news the FDA approved Ryoncil for GvHD. Anteris raised $138 million to list on the Nasdaq, and Percheron very honestly reported that avicursen failed to improve Duchenne muscular dystrophy.

And in great news missed by many who didn't understand its importance, Ghana approved Medicines Development for Global Health's moxidectin for river blindness. The US approved the Melbourne-developed drug in 2018, but Ghana is the first river blindness endemic country to approve it – meaning moxidectin will be used as a treatment and preventative. An excellent result for the not-for-profit MDGH.

Capital Raisings 2009 -2024



This year's capital raising is the best on record at $2,887 million. Far from doom and gloom, it has been an excellent year for biotechs.

"We'll all be rooned," they said. "Read the data," we said.

2024 AWARDS (Judges: David Langsam, Tim Boreham, Jamie Miller)

CEO OF THE YEAR

There are several serious candidates, but there was only one leader of the pack. The runners up were Dr Chris Behrenbruch at Telix, Dr Alan Taylor at Clarity, Cyclopharm's James McBrayer, Compumedics Dr David Burton, Dimerix's Dr Nina Webster, Immutep's Marc Voigt, Invion's Thian Chew and Oncosil's Nigel Lange.

As foreshadowed last year, Dr Sam Hupert and the team at Pro Medicus have forced Biotech Daily to promote them from the Biotech Daily Top-20 into the world of the Big Caps. It's $27 billion market capitalization has exceeded Cochlear's.

The **2024 CEO of the Year** is Dr Sam Hupert and the team at Pro Medicus.

The **2024 Chair of the Year** has been with-held this year. We can't just keep on giving it to David Williams and Paul Hopper every year.

The **2024 Kamikaze of the Year** was also hard fought. There were so many from which to choose. It is true that marijuana companies dominated the race to the bottom, but for complete ineptitude the award has to go to Genetic Technologies, for taking a very good idea and failing to commercialize it.

A long time ago, before founder Dr Mervyn Jacobson went to gaol for a year for insider trading, Genetic Technologies did genetic testing and some speak highly of that company.

But Dr Jacobson had set the course and sacking two good boards for not doing what they were told was just the beginning. The company then developed a so-called breast cancer test, which was more of a questionnaire, before revealing it had a new test that also worked for Black and Hispanic American women.

Hang on. You never told us that the test was exclusively for White women.

But that wasn't the end. Instead of charging $US4,000 including insurance reimbursement Genetic Technologies said it would sell the test directly to women … for $US200 a go. It was very hard to take the company seriously after that.

Finally, under chair Peter Rubinstein and chief executive officer Simon Morriss, Genetic Technologies has appointed the administrators.

Summer holiday publishing schedule

Biotech Daily will shut down for the long, hot, Australian Summer from tomorrow and be back on deck refreshed and recharged on Monday January 20, 2025.

Australia is on holidays, so DO NOT put out any announcements - that no-one will read anyway - for the next month. Go to the beach.

That said, we monitor all announcements and publish a Summer Holiday Catch-Up edition, highlighting any companies posting bad news after the market closes on Christmas Eve and New Year's Eve.

Biotech Daily would like to thank its team of advisers: Prof George Fink, Dr Stuart Garrow, and Michael Ibbott for invaluable wisdom, insights and cautions throughout the year.

Biotech Daily thanks star columnist Tim Boreham and deputy editors, Jamie Miller and Alex Langsam for all their superb work this year.

All errors through the year were the fault of the sacked sub-editors and none of the above.

We wish everyone an excellent Southern Hemisphere Summer break, Summer Solstice, Merry Christmas, Happy Chanukah and Hogmanay/New Year and see you all in 2025.

David Langsam, Editor

MESOBLAST

Mesoblast says the US Food and Drug Administration has approved Ryoncil for children aged two months and older for graft versus host disease (GvHD).

Mesoblast said the Ryoncil (remestemcel-L, previously known as MSC-100-IV) approval included adolescents and teenagers, with steroid-refractory acute graft versus host disease (SRaGvHD), a life-threatening condition with high mortality rates.

In 2018, Mesoblast said 41 of 55 children (74.5%) in its open-label, phase III trial of remestemcel-L for acute graft versus host disease survived to 100 days, and later said that 38 of 55 children (69%) survived to 180 days (BD: Jun 21, Sep 30, 2018).

In 2020, the FDA said it required a further trial of remestemcel-L and "recommended that [it] conduct at least one additional study in adults and/or children (BD: Oct 2, 2020).

Last year, Mesoblast said the FDA provided a second complete response letter requiring more data; and later said it expected to contract a pivotal trial (BD: Aug 4, Nov 15, 2023).

This year, the company said the 2018 phase III study data "appears sufficient" for a biologics' application for Ryoncil for paediatric steroid-refractory acute graft versus host disease and it resubmitted its application to the FDA (BD: Mar 26, Jul 9, 2024).

Today, Mesoblast said "Ryoncil's immunomodulatory effects, including inhibition of T-cell activation and secretion of pro-inflammatory cytokines, position the therapy for potential other indications in diseases with excessive inflammation … [and Ryoncil was] the only MSC therapy approved in the US for any indication".

In 2013, Mesoblast said it would acquire the mesenchymal stem cells assets of the Maryland-based Osiris for up to $US100 million in cash and scrip (BD: Oct 11, 2013).

Today, Duke University Medical Center's Dr Joanne Kurtzberg said that SRaGvHD was "a devastating condition with an extremely poor prognosis".

"From today we are able to offer Ryoncil, the first FDA-approved treatment which will be life saving for so many children and will have a great impact on their families," Dr Kurtzberg said.

Mesoblast said that 10,000 US patients a year underwent allogeneic bone marrow transplant, of which 1,500 were children, and about 50 percent develop aGvHD and almost half of those do not respond to steroids, the recognized first-line treatment.

Mesoblast chief executive Prof Silviu Itescu said the company was "very pleased that the FDA has granted approval of Ryoncil and are proud of the company's commitment to the GvHD community in bringing this important new treatment to children and families with no other acceptable options".

"With Ryoncil approval by FDA, Mesoblast has demonstrated the ability to bring the first [mesenchymal stromal cell] product to market," Prof Itescu said.

"We will continue to work closely with FDA to obtain approval of our other late-stage products, including Revascor for cardiovascular diseases and rexlemestrocel-L for inflammatory pain indications, as well as expanding the indications for Ryoncil in both children and adults with inflammatory conditions," Prof Itescu said.

Mesoblast said that Ryoncil would be available in the US at transplant centres and other treating hospitals "in the coming weeks".

An attached media release from the FDA said that remestemcel-L had fast track, orphan drug and priority review designations.

The FDA said that the recommended dose was 2.0 x 10^6 MSC/kg body weight per intravenous infusion given twice a week for four weeks for a total of eight infusions, administered at least three days apart.

Treatment may be continued based on response at 28 days.

In late November, Mesoblast's market capitalization surpassed $2.0 billion.

Mesoblast was up $1.07 or 54.0 percent to $3.05 with 57.8 million shares traded.

PETER MACCALLUM CANCER CENTRE

The Peter MacCallum Cancer Centre says Crispr gene editing can “silence cancer-causing gene mutations” known to cause pancreatic, colorectal and lung cancers.
Earlier this year, the Peter MacCallum said it was using clustered regularly interspaced short palindromic repeats, or Crispr, technology to develop “rapid personalized cancer treatments by ‘cutting out’ disease-causing RNA” (BD: Jul 3, 2024).
Today, a media release from Melbourne’s Peter MacCallum Cancer Centre said it had shown it was possible to silence the KRAS G12, NRAS G12D, and BRAF V600E gene mutations known to drive aggressive pancreatic, colorectal and lung cancers.
The Centre said Crisper was a tool for targeting and disabling or editing specific DNA in cells and using the Cas13 protein allowed Crispr to target RNA, the downstream information sent by DNA, rather than DNA itself.
The Peter MacCallum Centre said the mutations silenced were single-nucleotide variants (SNV), small changes in the genetic code that fuel uncontrolled cell growth.
The Centre said the researchers were able to selectively degrade RNA messages from the mutant genes while having no effect on related healthy genes.
The Peter MacCallum Centre said the research was led by Dr Mohamed Fareh and that more work was “needed before this novel method could be tested in people”.
The Centre said the paper, titled ‘Principles of Crispr-Cas13 mismatch intolerance enable selective silencing of point-mutated oncogenic RNA with single-base precision’ was published in the Science Advances, with the full study at: https://bit.ly/3Bzb8zQ.
Dr Fareh said Crispr-Cas13 had the potential to be a “precise, mutation-specific drug”.
“With further development, this platform could transform the way we treat cancers driven by hard-to-target mutations,” Dr Fareh said.
“The precision and adaptability of this system also opens new doors to personalized cancer treatments tailored to an individual’s unique genetic profile,” Dr Fareh said.
“These SNVs have been notoriously difficult to target with traditional drugs,” Dr Fareh said. “By introducing strategic mismatches in the Crispr guide RNA, we successfully reprogrammed Crispr-Cas13 to selectively degrade mutant RNA transcripts while sparing the normal, unmutated versions expressed in healthy cells.”
Dr Farah said the approach overcame the limitations of earlier therapeutic applications of Crispr and achieved one of a feature of true, targeted drugs, blocking only the mutated forms of the genes.

ORTHOCELL

Orthocell says it has submitted a 510(k) application to the US Food and Drug Administration for Remplir for peripheral nerve repair.
Earlier this month, Orthocell said it would file “positive” regulatory study data to the FDA showing its Celgro-based Remplir was “safe and effective” for nerve repair, in 72 rats with sciatic nerve injury (BD: Dec 2, 2024).
Today, the company said based on the expected 90 calendar day review process for US FDA 510(k) submissions and allowing for the festive period closures, it expected market clearance for Remplir by “late March or early April 2025”.
The company said it was “well advanced” for its preparations with the appointment of US sales and medical affairs staff for the launch and sales “immediately post clearance”.
Orthocell said Remplir was already approved and selling in Australia, New Zealand and Singapore with “rapidly growing sales, and an increasing number of surgeons using and endorsing its unique repair qualities in clinical practice”.
Orthocell fell half a cent or 0.4 percent to $1.25 with 2.6 million shares traded.

CERTA THERAPEUTICS, OCCURX, BRANDON CAPITAL

Melbourne's Certa says it has acquired Occurx to develop GPR68 targeting treatments for fibrotic diseases, including focal segmental glomerulo-sclerosis (FSGS).

A media release from Melbourne's Certa said both companies were founded by Prof Darren Kelly, and that the acquisition would consolidate its pipeline with Occurx's to include two phase II and one phase I clinical candidate.

The company said its lead candidate was asengeprast, or FT011, an oral treatment for systemic sclerosis and it was also developing CTA382 for chronic kidney disease, while Occurx had completed a phase I trial of OCX063 for focal segmental glomerulo-sclerosis.

In 2022, Occurx said it had raised $16 million from Brandon Biocatalyst and Uniseed to progress to a phase I clinical trial of its OCX-063 oral therapy for inflammation and fibrosis in healthy volunteers (BD: Sep 5, 2022).

Last year, Certa said its 30-patient, double-blinded, phase II trial showed that 400mg oral FT011 had a "clinically meaningful improvement" in 60 percent of systemic scleroderma patients (p = 0.019) (BD: Nov 16, 2023).

Today, the company said asengeprast began as an asset of Fibrotech, which was sold to Shire in 2014 before Shire's eventual acquisition in 2018 by Takeda, when Brandon Capital and Uniseed reinvested in the asengeprast program by forming Certa; and to develop GPR68-targeting ophthalmology assets by forming Occurx.

Certa said it and Occurx had raised $US30 million ($A44.5 million) in six years to advance the development of these assets and that Certa would "now seek to raise a series B to continue the clinical development of its pipeline and expand its operations".

Today, Certa chair and Brandon Capital managing partner Dr Chris Nave said that "bringing together the assets and expertise of Certa and Occurx under one company creates a powerful platform to advance transformative therapies for fibrotic diseases".

"This consolidation reinforces Certa's leadership in the GPR68 field and its commitment to developing innovative treatments with a promising clinical-stage pipeline of GPR68 antagonists," Dr Nave said.

Both Certa and Occurx are private companies.

STARPHARMA HOLDINGS

Starpharma says it has "positive feedback" from the US Food and Drug Administration on a regulatory approval pathway for its DEP-SN38, formerly DEP-irinotecan.

Last month, Starpharma said its dendrimer enhanced product (DEP)-SN38 showed "promising efficacy" for colorectal cancer and ovarian cancer (BD: Nov 21, 2024).

Today, the company said the FDA agreed that DEP-SN38 could be considered for FDA fast-track designation in platinum-resistant ovarian cancer and that a 505(b)(2) regulatory approval pathway was appropriate.

Starpharma said the FDA indicated that DEP-SN38 "may qualify for accelerated approval based on an interim analysis of early surrogate endpoints from the proposed phase II/III clinical trial program".

The company said final outcomes would "depend on the results of these studies and the overall data package; however, this accelerated approval could provide early access to DEP-SN38 for patients with platinum-resistant ovarian cancer".

Starpharma chief executive officer Cheryl Maley said the FDA feedback was "an important milestone, supporting the [investigational new drug] submission, which a partner can advance through further development, registration and commercialization".

Starpharma was up 0.3 cents or 3.1 percent to 10 cents.

EBR SYSTEMS

EBR says its manufacturing pre-approval inspection with the US Food and Drug Administration has been scheduled for the week beginning January 6, 2025.
Earlier this year, EBR said it filed the final module for its Wise cardiac re-synchronization therapy system to the FDA and its 100-day pre-market approval meeting with the FDA had been scheduled for December 20, 2024 (BD: Aug 29, Dec 6, 2024, 2024).
Today, the company said the pre-approval inspection would confirm its “manufacturing, processing and packing procedures comply with quality system regulations, and that EBR’s facility can consistently produce devices that meet the approved specifications”.
EBR was up 12.5 cents or 14.5 percent to 98.5 cents.

BLINKLAB

Blinklab says the US Food and Drug Administration has confirmed the study design and data requirements needed for a 510(k) clearance of its autism diagnostic.
Last month, Blinklab said a 441-child study showed its smartphone platform “effectively identifies … children with autism” with 91 percent sensitivity and 85 percent specificity, and chair Brian Leedman said the results confirmed “the accuracy of our … test for autism” and provided confidence for a forthcoming FDA trial (BD: Nov 19, 2024).
Today, the company said it would conduct an initial study of 100 patients followed by a main study with up-to 1,000 children with autism between two and 11 years of age.
Blinklab said both phases of the study would be prospective, double-blinded and involve comparing its Dx1 smartphone application with the DSM-5, or Diagnostic and Statistical Manual of Mental Disorders, Fifth Edition, based diagnostic standards.
The company said it expected to complete both studies within 16 months and would submit a 510(k) application to the FDA following the trial.
Mr Leedman said the outcome “was a significant milestone”.
“With this guidance from the pre-submission meeting, we are confident in our study design and ability to bring Blinklab Dx1 to market,” Mr Leedman said.
Blinklab was unchanged at 24.5 cents.

MAYNE PHARMA

Mayne Pharma says its $38 million settlement of a shareholder class action has been approved by the Supreme Court of Victoria, with no admission of liability.
In 2021, Mayne Pharma said it had been served with a class action by Phi Finney McDonald for the plaintiff and on behalf of all persons who acquired an interest in shares and/or American depositary receipts between November 24, 2014 and December 15, 2016, to be held at the Supreme Court of Victoria (BD: Aug 3, 2021).
In July, the company said it would pay $38 million to settle a class action related to “alleged misleading or deceptive conduct and breaches of continuous disclosure obligations in respect of alleged anticompetitive conduct in the US that was previously the subject of investigations by the US Department of Justice and the Office of the Attorney General in the State of Connecticut” (BD: Jul 2, 2024).
At that time, Mayne Pharma said $4.7 million of the settlement would be covered by insurance, with the remainder to be paid from its cash reserve, and it held cash and marketable securities of $146.8 million at December 31, 2023.
Today, the company said that under the terms of the settlement “the claims of the plaintiff and group members are dismissed without admission of liability by the company”.
Mayne Pharma fell 10 cents or two percent to $4.85.

LTR PHARMA

LTR says its Spontan nasal spray for erectile dysfunction will be supplied by Men's Health Downunder "Australia's largest men's health pharmacy clinic network".
In August, LTR said it dosed the first erectile dysfunction patients with its Spontan nasal spray version of vardenafil, marketed by Bayer as Levitra, under the Australian Therapeutic Goods Administration's special access scheme (BD: Aug 5, 2024).
Today, the company said the agreement allowed it to access Men's Health Downunder's network of pharmacists including general practitioners, urologists and sexual health clinics and that the pharmacy clinic network serviced more than "1,000 patients annually through specialist urological health support services".
LTR did not disclose the commercial terms of the agreement.
LTR chair Lee Rodne said that the deal was "another significant milestone in Spontan's commercialization journey".
"By partnering with Australia's largest men's health pharmacy clinic, we continue to broaden access to Spontan while maintaining our commitment to patient care through qualified healthcare professionals," Mr Rodne said.
LTR was up one cent or 1.2 percent to 85 cents.

FIREBRICK PHARMA

Firebrick says it has signed a six-month extension to its consulting agreement with Keith Shortall for the commercialization of its Nasodine nasal spray in Singapore.
In September, Firebrick said it hired Singapore consultant Keith Shortall to help establish distribution for Nasodine in Singapore and would pay him consulting fees and 1.5 million options for an initial 50-days of work to December 31, 2024 (BD: Sep 9, 2024).
Today, the company said the extended agreement was effective from January 1, 2025 and ended June 30, 2025, unless further extended by mutual agreement.
Firebrick said Mr Shortall would receive a further 1.5 million options during the extended term, in addition to his consulting fees, exercisable at nine cents each within three years and vesting on sales and partnership milestones.
Firebrick was unchanged at 5.3 cents.

LITTLE GREEN PHARMA

Little Green says it welcomes changes to Western Australia's medical marijuana prescribing and dispensing regulations.
Little Green said the revised regulations allowed Western Australian patients to be prescribed medical marijuana issued by interstate telehealth clinics or prescribers dispensed at local Western Australia pharmacies "eliminating the previous requirement for interstate dispensing and postal delivery".
The company said under the changes Western Australia prescribers were no longer required to obtain Western Australia state health chief executive officer's authorization to prescribe medicinal cannabis products for eligible patients.
Little Green said the regulatory updates aligned Western Australia with "national medicinal cannabis guidelines while maintaining robust safeguards for patients and public safety".
The company said it was headquartered in Western Australia and that the changes "significantly benefit" its operations.
Little Green fell half a cent or 3.85 percent to 12.5 cents.

IMUGENE

Imugene says it has received $11 million from the Australian Taxation Office under the Federal Government's Research and Development Tax Incentive program.
Imugene said the incentive related to research and development expenditure for the year to June 30, 2024 and was expected to be received in January 2025.
Imugene fell 0.2 cents or 5.3 percent to 3.6 cents with 22.4 million shares traded.

RADIOPHARM THERANOSTICS

Radiopharm told the ASX it became aware of pre-clinical RAD-402 data on December 5 and "considered that it had an obligation" to announce it late on December 9, 2024.
In an aware query, the ASX asked Radiopharm whether it believed the information announced to the ASX on December 10, 2024, titled 'Completion of pre-clinical data package for RAD 402' was material and when it became aware of the information.
The ASX noted the company's share price rose 50.0 percent from 2.8 cents at the close of trading on December 5 to a high of 4.2 cents on Monday December 9, 2024, but did not mention an increase in the volume of shares traded.
Radiopharm said its trading on the Nasdaq "had shown a high volume of trading and it was assumed that the ASX share price and volume volatility followed on from the earlier Nasdaq trading".
The company said prior to its decision that it was obliged to release the information it "was assessing the information based on Code of Best Practice for Reporting by Life Science Companies to determine if there was adequate information to warrant disclosure as price sensitive".
Radiopharm said "on reflection, there should have been a further enquiry within the company, based on an ASX triggered phone call, to ensure there wasn't any other information being considered for market release'.
Radiopharm fell 0.2 cents or 8.7 percent to 2.1 cents with 6.2 million shares traded.

ACTINOGEN MEDICAL

BVF Partners says it has reduced its substantial shareholding in Actinogen from 247,334,680 shares (8.40%) to 228,334,680 shares (7.29%).
The San Francisco-based BVF said with Mark Lampert it sold shares on-market between November 12 and December 4, 2024, with the single largest sale 2,451,553 shares on November 20 for $62,879, or 2.6 cents a share.
Actinogen fell 0.1 cents or 3.85 percent to 2.5 cents with 7.8 million shares traded.