

NSW-RPRN Capabilities

Production Pillar
Services and Support Pillar

NSW-RPRN Symposium
23-24 March 2023
Joshua Peterson
rprn@unsw.edu.au



Production Pillar

Prime the RNA therapeutics production industry in NSW by providing researchers access to products at sufficient quality and scale for use in pre-clinical studies.

Nucleotides/lipids

Richard Payne (USyd)
Christopher McErlean (USyd)

Synthetic RNA

Albert Fahrenbach (UNSW)
Pall Thordarson (UNSW)

Plasmid DNA

Christopher Marquis (UNSW)

mRNA

Scott Cohen (CMRI/USyd)
Christopher Marquis (UNSW)

Lipid nanoparticles

Maria Kavallaris (CCI/UNSW)
Joshua McCarroll (CCI/UNSW)
Pall Thordarson (UNSW)

Support/Services Pillar

Support the establishment of an RNA-based therapeutics ecosystem in NSW and beyond by ensuring the successful execution of NSW-RPRN activities.

Sequencing/genomics

Helena Mangs (Ramaciotti Centre, UNSW)
Vitali Sintchenko (WIMR/USyd)
Rebecca Rockett (WIMR/USyd)

PC3 lab/animals for SARS-CoV-2

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RNA Accelerator

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The **RNA Accelerator** gives clients access to RNA-based therapeutics and key intermediates at a quality and scale suitable for pre-clinical studies.



- Oct 2021 – Started major equipment procurement, facility design & hiring
- Nov 2022 – ISO 9001 Certified
- Jan 2023 – moved into new facility
- Today
 - ~15 staff
 - Purpose built facility > 250 m² including Clean Room
 - Manufactured >100 batches of RNA-based therapeutics



Pilot-scale manufacture and design and development of new processes for RNA based therapeutics and other products for pre-clinical studies.

Synthetic RNA

- siRNA up to gRNA (5 – 80/100 nt)
- Production up to ~1 g siRNA

pDNA

- Construct design
- Cell banking
- Production to 10 L scale (~50 mg)

mRNA/saRNA

- IVT production to 10 L scale (~1 g)

NP Formulation

- NP formulation up to 60 mL
- LNP and dendrimer formulations

Quality Control

- Analytical procedures based on USP guidelines and TGA testing
- Special instrument capabilities:
 - HPLC-MS Orbitrap 120
 - Analysis and sequencing of synthetic RNA
 - HPLC-MS based mRNA assays (capping efficiency, etc.)
 - HPLC-CAD
 - Analysis of NP formulations
- Access to ISO 17025 certified sequencing and characterisation in Ramaciotti Centre for Genomics
- Access to ISO 9001 certified local production of enzymes, proteins from UNSW Recombinant Products Facility (RPF)

Contact Us



unswrna.institute



rna@unsw.edu.au

The **RNA Accelerator** is an TIA/NCRIS* node for RNA therapeutics

- Applications for funding support are available through the Pipeline Accelerator scheme (current round of applications close 28 April)

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Ramaciotti Centre for Genomics

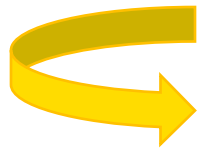
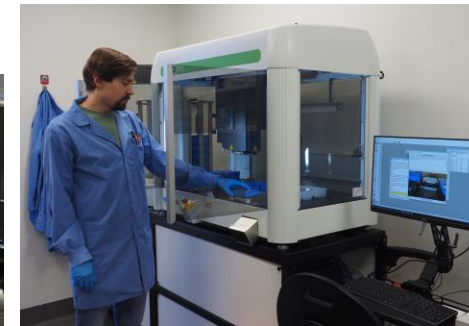
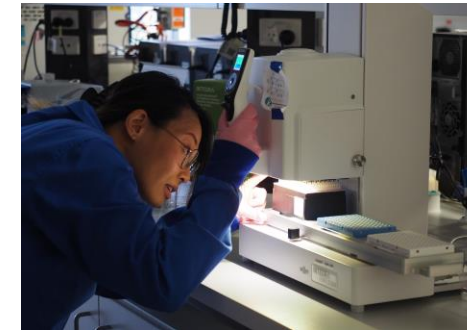
- National infrastructure facility – genomics core lab
- Largest, most comprehensive genomics facility at an Australian University
- Provide sequencing, microarray and other genomic services
- >1,000 projects per year, >85,000 samples per year
- NATA accredited to ISO/IEC 17025:2017
- 23 staff members
- Personalised approach
- Experienced, Centre established 23 years ago



Ramaciotti Centre for Genomics

core services:

- Sanger sequencing
- NGS long read: Oxford Nanopore (ONT) GridION & PromethION
- NGS short read: Illumina NovaSeq X Plus (to arrive soon), NovaSeq 6000, NextSeq 1000, MiSeq's, iSeq 100. MGI G400
- Genotyping by microarray: Thermo Fisher Axiom & Illumina Infinium arrays (HT)
- Single cell RNA-seq: through UNSW Cellular Genomics Futures Institute (UNSW & the Garvan Institute of Medical Research)



- RNA/DNA/modifications
- Whole transcriptome/genome or targeted sequences



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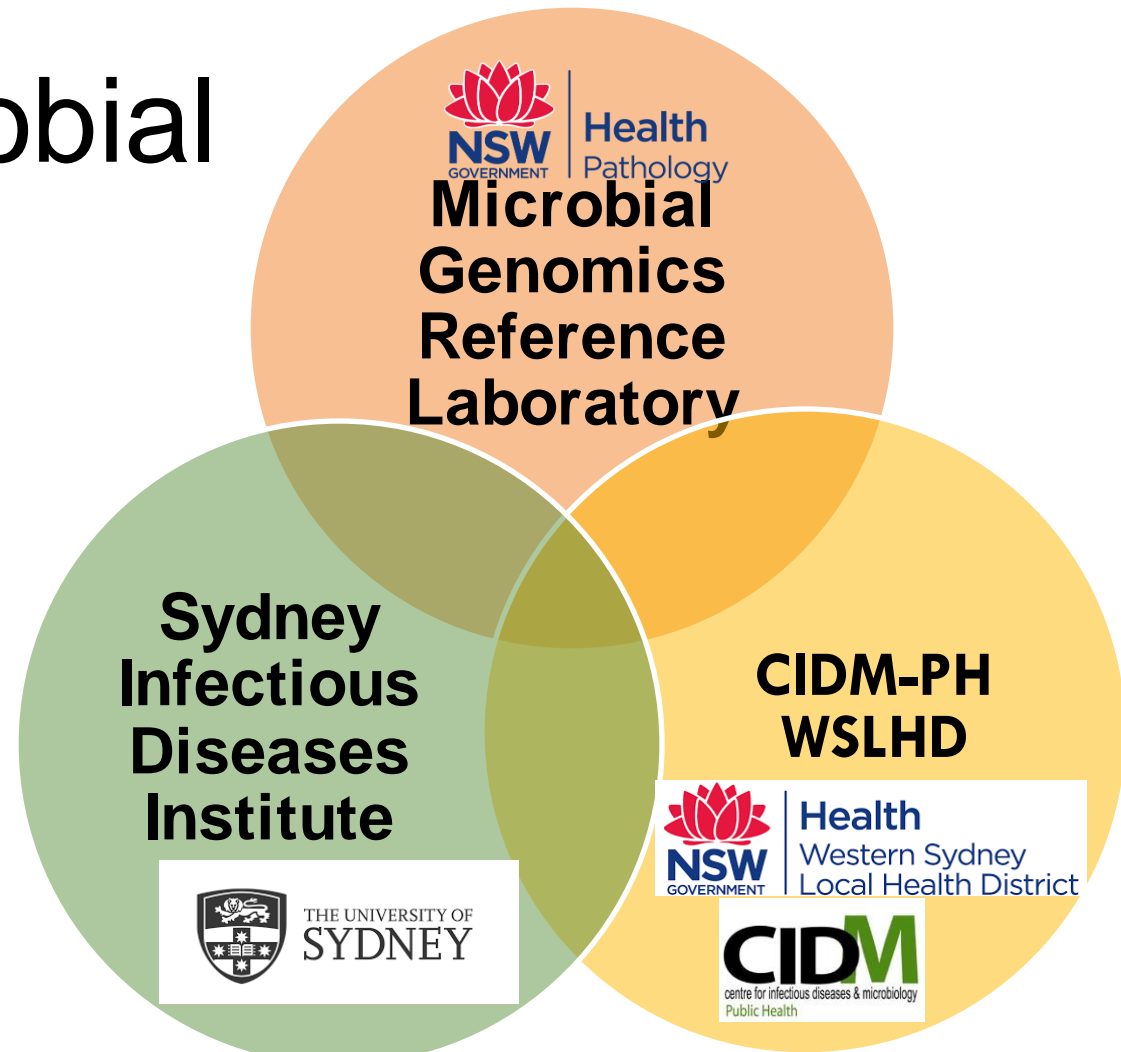
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Pall Thordarson (UNSW)



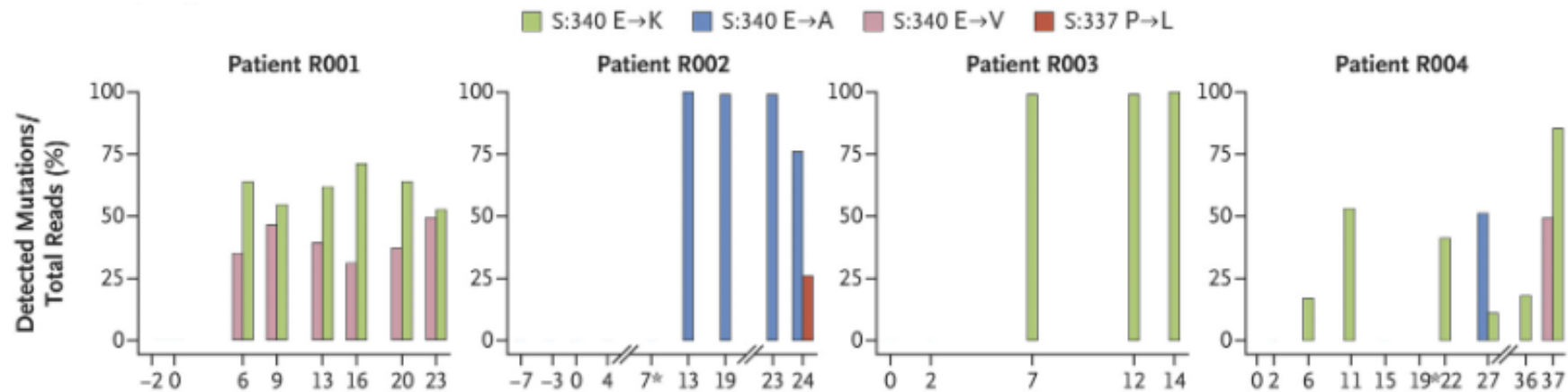
Applied microbial genomics

Clinical genomics
Specialist bioinformatics
Targeted metagenomics
Meta-transcriptomics



Genomics and Clinical Microbiology

- Of 100 patients to receive sotrovimab infusions eight patients were persistently SARS-CoV-2 positive (>10 days) and had serially collected specimens
- Four of eight patients developed resistance conferring mutations within the sotrovimab binding site



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Australian Proteome Analysis Facility (APAF)

23 MARCH 2023

Dr Belinda Schiller

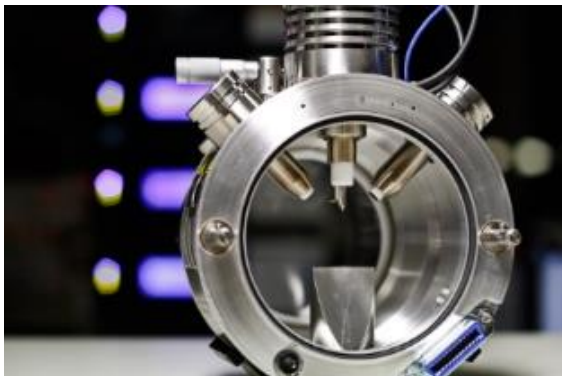


CAPABILITIES

AUSTRALIAN PROTEOME ANALYSIS FACILITY (APAF)

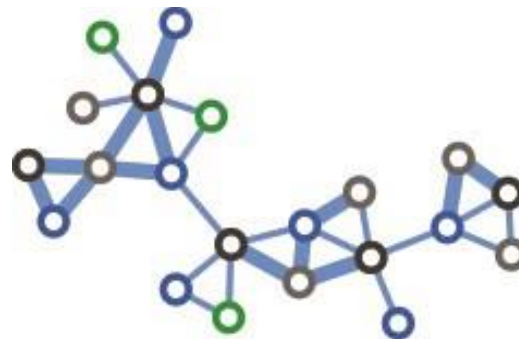
- The Australian Proteome Analysis Facility (APAF) is a service and research facility within Macquarie University
- Research support and services across all sectors including biomedical, life sciences, environmental, food and agriculture, for academia, industry, and government organisations.

MASS SPECTROMETRY



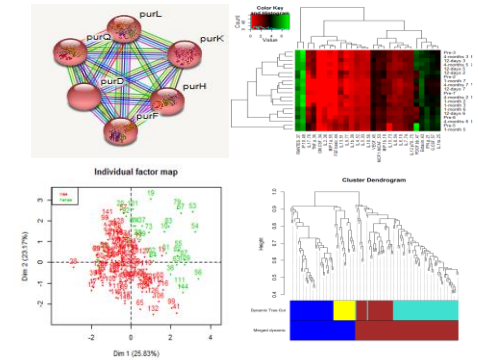
POST TRANSLATIONAL MODIFICATIONS (PTMS)

PROTEIN ANALYSIS AND CHARACTERISATION



PROTEIN INTERACTIONS

BIOINFORMATICS



INTEGRATED 'OMICS

OVERVIEW

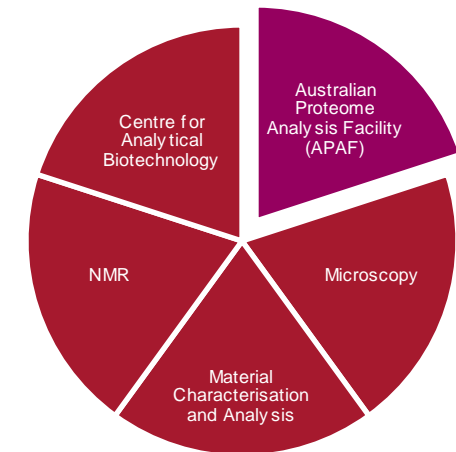
AUSTRALIAN PROTEOME ANALYSIS FACILITY (APAF)

- APAF provides an accessible range of world class instrumentation and specialist technical support for training, teaching, and research

Research support and service facility

- Part of the shared research infrastructure within Macquarie University
- Access for internal and external users
- Training and teaching support
- Research support and collaborations
- Method development
- Quality control testing
- NATA accredited services

MACQUARIE ANALYTICAL AND FABRICATION FACILITY (MAFF)



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A robotic arm is shown in a laboratory setting, performing high-throughput screening. The arm is white and blue, with a red warning label. It is holding a blue tray and is positioned over a work area. The background is a blurred laboratory environment with blue lighting. The text "High-throughput screening in the ANU Centre for Therapeutic Discovery" is overlaid on the image in white.

High-throughput screening in the ANU Centre for Therapeutic Discovery



Australian
National
University

ANU Centre for Therapeutic Discovery

What we offer:

- Open to all research disciplines
- 'Collaborative' and fee-for-service screening models
- Consultation about screen design and assay workflows
- Assistance with grant applications (screening aims)
- Screening of biological and chemical agents
 - arrayed CRISPR, RNAi and drug libraries
- General screening consumables and reagents for purchase
- Boutique microplate applications and pilot experiments using automation



ANU Centre for Therapeutic Discovery

For more information

Dr Ameer George

Manager, ANU Centre for Therapeutic Discovery

Email: amee.george@anu.edu.au

Phone: (02) 6125 3086

Web: jcsmr.anu.edu.au/research/centres/actd

Email: actd.brf@anu.edu.au

Twitter: @ANU_Therapeutic



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University of Technology Sydney
Biologics Innovation Facility



David Rickards

David.Rickards@uts.edu.au

02 9514 4775

Training/GLP/GMP/clinical support – UTS BIF

- Clean room management;
Environmental monitoring, QMS documentation, facility operations
- Single-use equipment for manufacture;
IVT, chromatography, UF/DF, encapsulation, filling
- Adaptable for development of new technology in the process;
Monolith columns
- Expertise and strong partnerships with key suppliers of RNA production technology



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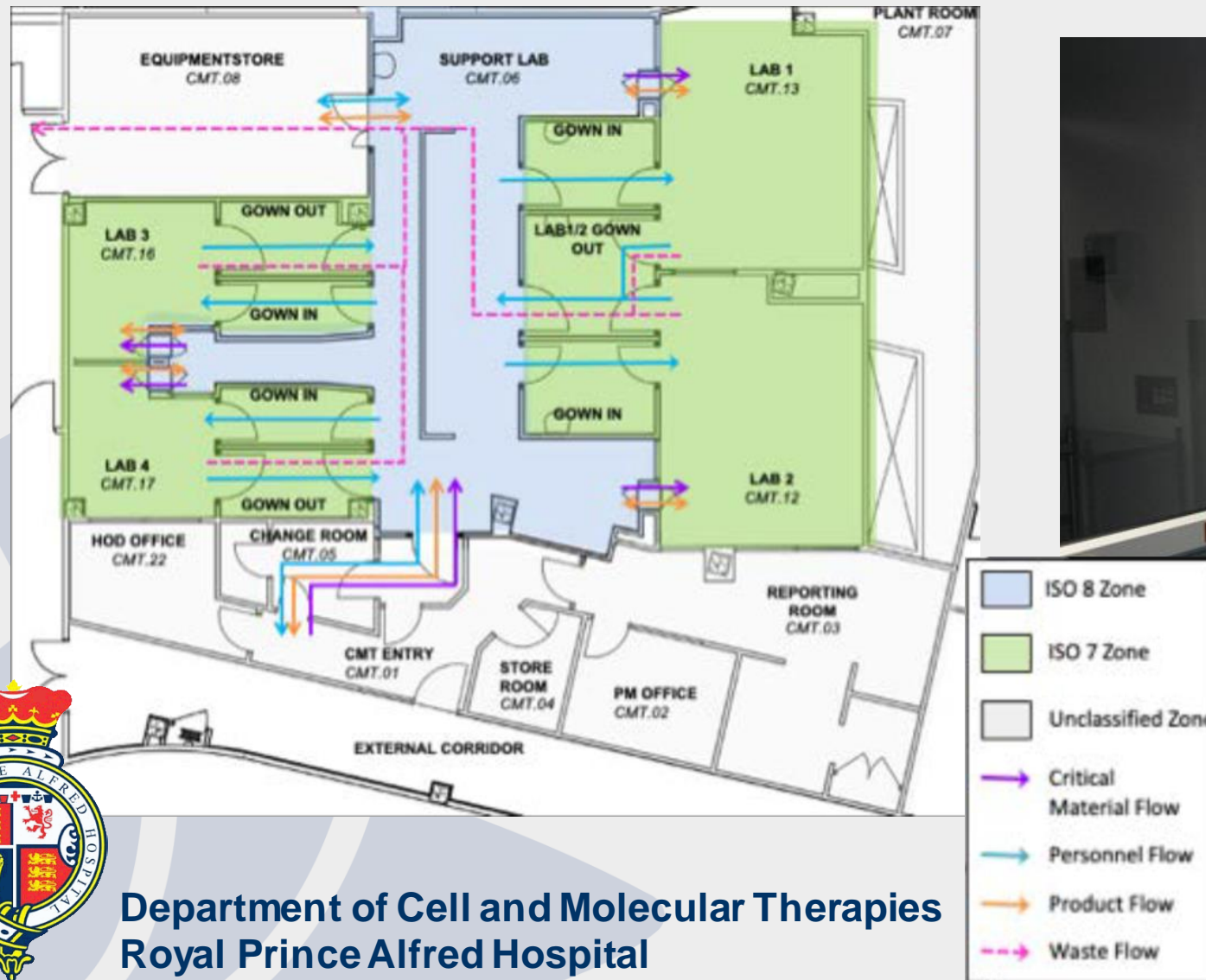
Cell & Molecular Therapies (RPAH/Centenary/USyd)

Management

Pall Thordarson (UNSW)



Cell & Molecular Therapies, Royal Prince Alfred Hospital

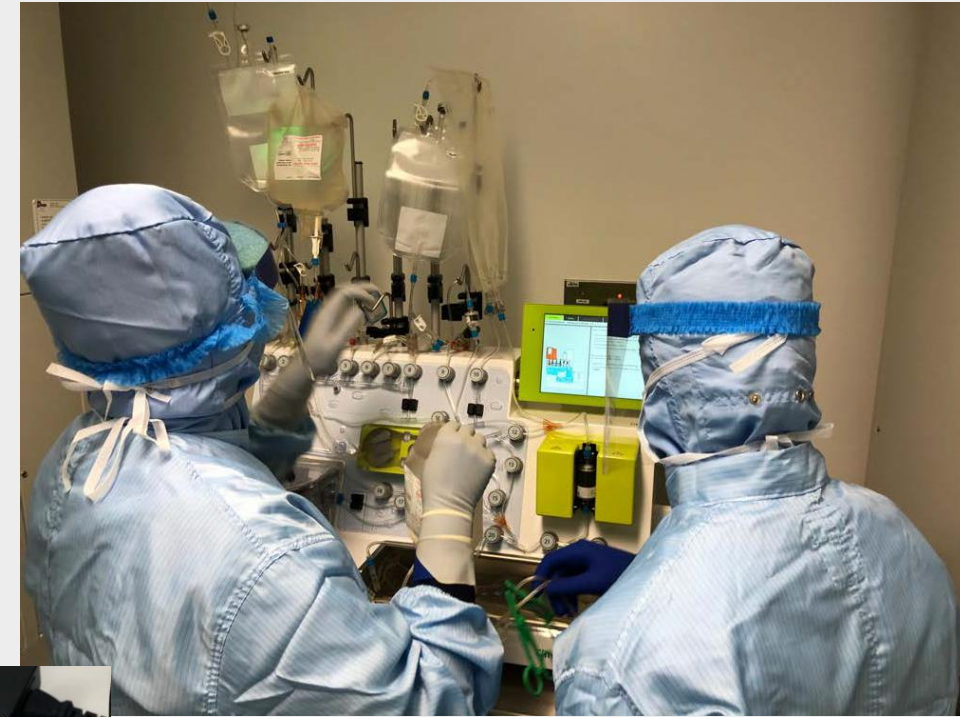


Department of Cell and Molecular Therapies
Royal Prince Alfred Hospital



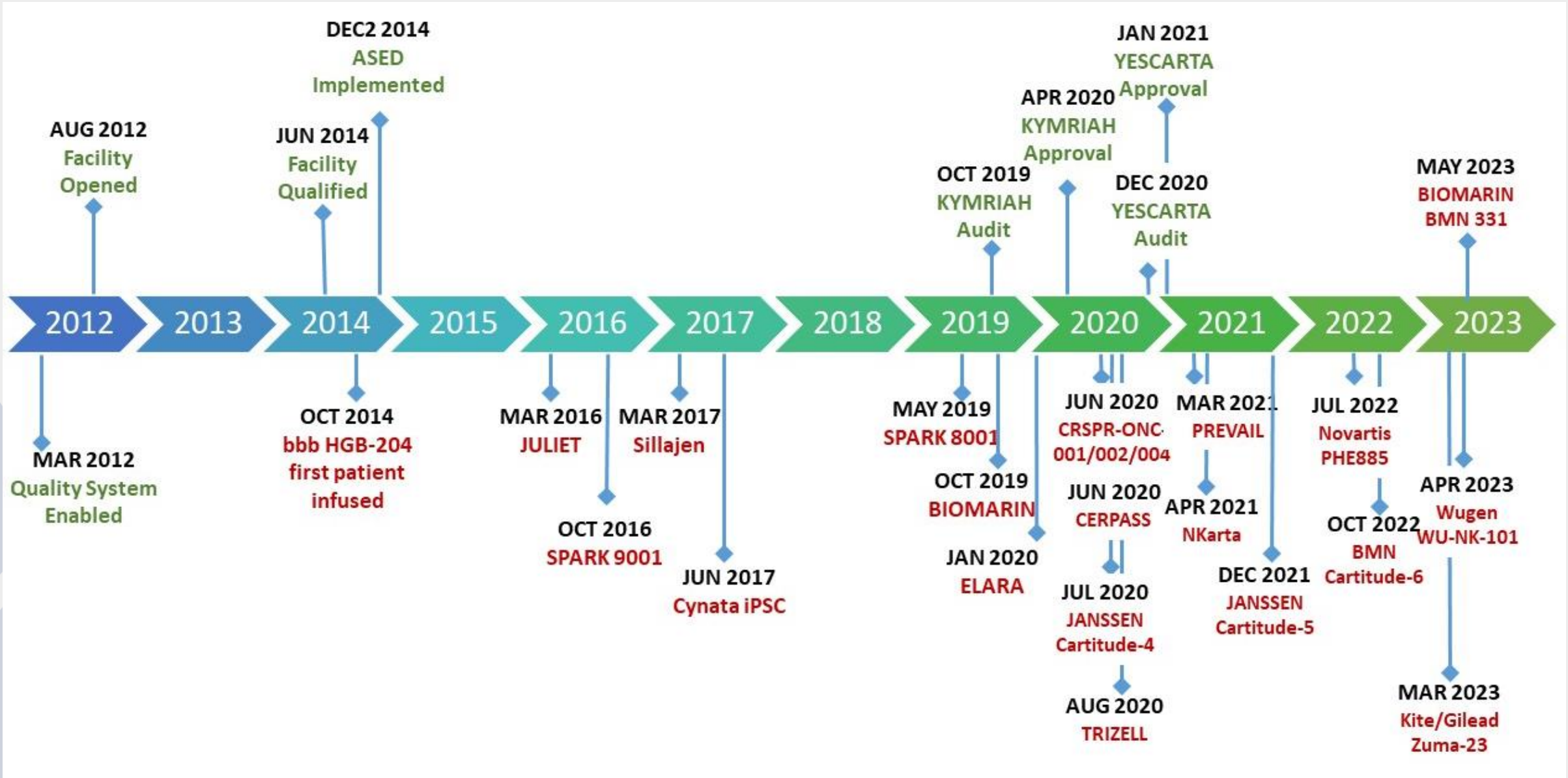
Health
Sydney
Local Health District

Cell & Molecular Therapies: Manufacturing



Health
Sydney
Local Health District

Cell & Molecular Therapies: Timeline



Cell & Molecular Therapies: Pioneering Cell and Gene Therapies

Clinical Trial > [N Engl J Med. 2021 Nov 18;385\(21\):1961-1973. doi: 10.1056/NEJMoa2104205.](#)

Multiyear Factor VIII Expression after AAV Gene Transfer for Hemophilia A

Clinical Trial > [N Engl J Med. 2017 Dec 7;377\(23\):2215-2227. doi: 10.1056/NEJMoa1708538.](#)

Hemophilia B Gene Therapy with a High-Specific-Activity Factor IX Variant

Comparative Study > [Nat Med. 2007 Apr;13\(4\):419-22. doi: 10.1038/nm1549.](#)

Epub 2007 Mar 18.

CD8(+) T-cell responses to adeno-associated virus capsid in humans

Clinical Trial > [Nat Med. 2006 Mar;12\(3\):342-7. doi: 10.1038/nm1358.](#) Epub 2006 Feb 12.

Successful transduction of liver in hemophilia by AAV-Factor IX and limitations imposed by the host immune response

Clinical Trial > [N Engl J Med. 2018 Apr 19;378\(16\):1479-1493. doi: 10.1056/NEJMoa1705342.](#)

Gene Therapy in Patients with Transfusion-Dependent β -Thalassemia

nature
medicine

2020

LETTERS

<https://doi.org/10.1038/s41591-020-1050-x>



Production, safety and efficacy of iPSC-derived mesenchymal stromal cells in acute steroid-resistant graft versus host disease: a phase I, multicenter, open-label, dose-escalation study



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