



**CSL**

**CSL**  
2024 Research Acceleration Initiative

November 2023  
Research External Innovation Europe

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# AGENDA



Overview of CSL



CSL Research  
Acceleration Initiative



Benefits of  
collaborating with CSL



CSL's core  
Therapeutic Areas



Areas of interest for  
collaboration



Questions



# Overview of CSL





# Our Businesses

# CSL

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graph TD; CSL[CSL] --- CSL_Behring[CSL Behring]; CSL --- CSL_Seqirus[CSL Seqirus]; CSL --- CSL_Vifor[CSL Vifor];
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## CSL Behring

Biotherapies & Rare Disease

## CSL Seqirus

Vaccines

## CSL Vifor

Iron Deficiency & Nephrology

## Driven by **Our Promise**

\* CSL will follow the required legal processes of formally changing the names for Vifor Pharma and Seqirus entities in due course.

# CSL at a Glance

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40+ 

**Countries** of operations  
around the world

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US\$13.3 

**Billion** in annual revenue

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US\$5.1 

**Billion** in R&D investments in the last  
5 years to advance product pipeline

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32,000+ 

**Employees** around the world

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2000+ 

**R&D** employees

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340+ 

**Plasma collection** centres across  
China, Europe and North America

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# Top 25 Biotech Companies of 2023

Rank	Company	Ticker Symbol	Market Cap (US\$ Billion)
1	Novo Nordisk	NOVO-B (CPH)	371.64
2	Thermo Fisher Scientific	TMO (NASD)	210.51
3	Amgen	AMGN (NASD)	126.31
<b>4</b>	<b>CSL Ltd</b>	<b>CSL (ASX)</b>	<b>101.4 B</b>
5	Gilead Sciences Inc	GILD (NASD)	99.98
6	Vertex Pharmaceuticals	VRTX (NASD)	88.83
7	Regeneron Pharmaceuticals	REGN (NASD)	87.64
8	Daiichi Sankyo	4568 (TOKYO SE)	69.55
9	Moderna	MRAN (NASD)	50.56
10	Jiangsu Hengrui Medicine Co Ltd	600276 (SHSE)	46.31
11	Chugai Pharmaceutical	4519 (TOKYO SE)	45.86
12	Biogen	BIIB (NASD)	44.69
13	Lonza	LONN (SWX)	44.33
14	Samsung Biologics	207940 (KRX KE)	44.12
15	Agilent Technologies	A (NYSE)	40.08
16	Seagan	SGEN (NASD)	37.5
17	Illumina	ILMN (NASD)	30.79
18	WuXi App Tec	603259 (SSEC)	29.33
19	Sun Pharmaceutical Industries	SUNPHARMA (NSE)	28.73
20	BeiGene	BGNE (NASD);06160 (HKE); 688235 (STAR SSEC)	27.50



# CSL's Key Global R&D Locations



Expanding footprint and alliances in leading biomedical locations centres

**>2,000+** employees in 10 countries



# Research Acceleration Initiative



# CSL's Research Acceleration Initiative

**Objective:** to build relationships with entrepreneurial researchers and fastrack discovery of innovative medicines that address unmet needs

**Why?** Early collaborations with high quality academic partners are key to building a sustainable pipeline

CSL's RAI provides a differentiated approach to partnering:

- ✓ Up to USD \$400,000 funding over 2 years
- ✓ CSL scientific champion assigned to each project
- ✓ Focused on early-stage projects
- ✓ Simple and fast 300-word initial application
- ✓ Clear and transparent timelines



## CSL Research Acceleration Initiative


Seeking Expressions of Interest from Research Organizations

CSL is a leading global biotech company that develops and delivers innovative biotherapies to help people living with life-threatening medical conditions live full lives.

CSL's **Research Acceleration Initiative** aims to fast-track discovery of innovative biotherapies through partnerships between CSL and global research organizations. These partnerships provide funding and access to industry experts for scientists working on novel biotherapeutic strategies in CSL's therapeutic areas.

**Expressions of interest** are sought from Business Development / Commercialization representatives across global research organizations that wish to participate in the 2024 CSL Research Acceleration Initiative.

The 2024 Research Acceleration Initiative will focus on innovative research projects that address unmet medical needs and are aligned with CSL's **Therapeutic Areas** and scientific **Platforms**:



To register your research organisation please email [RAI@csl.com.au](mailto:RAI@csl.com.au) by 15<sup>th</sup> December 2023

# CSL has invested in 30+ RAI partnerships since 2019

"We had a **stellar experience participating in the CSL RAI process**. The information material, informational webinars, and access to the program team for Q&A was well received by our faculty"

RAI 2023 participant  
University of Pittsburgh

"It has been a great pleasure to collaborate with our colleagues at CSL. The Research Acceleration Initiative (RAI) is an **outstanding platform that helps bridge the academic world with industry.**"

RAI 2021 awardee  
Justus-Liebig-Universität Giessen

"**CSL Behring has proven to be an exceptional collaborator**, fostering a scientifically focused partnership marked by open scientific exchange and generosity. Their extensive research expertise has consistently enriched our collaborative efforts"

RAI 2021 awardee  
Klinikum der Universität München (KUM)

"...the webinar session was very useful because it **clearly indicated which areas CSL was interested in funding**, thereby allowing me to focus my thoughts on them."

RAI 2022 awardee  
Nanyang Technological University

"Peerless experience – **timely, transparent, actionable communication.**"

RAI 2023 participant  
University of British Columbia

"...the opportunity to address **feedback from CSL and to refine the project was particularly valuable**"

RAI 2023 participant  
The University of Adelaide

"Well-designed, easy and clear process. **Highly engaged and highly responsive to all questions** and provided well-contemplated and customised feedback."

RAI 2023 participant  
University of Toronto

"The types of projects CSL were looking for was made very clear, the process of submitting an application was easy and **did not require excessive time or effort.**"

RAI 2023 participant  
Auckland UniServices Ltd

# SEVEN NEW CSL RESEARCH

# 7

## ACCELERATION INITIATIVE AWARDEES ANNOUNCED

**Dr Laurent Martinez**

Institute of Cardiovascular and Metabolic Diseases (I2MC), IHU HealthAge, INSERM / University of Toulouse, France

**Prof. Delphine Borgel**

INSERM - APHP - Université Paris SACLAY, France

**Prof. Denis Vivien**

INSERM / Caen Normandie University Hospital, France

**Research Director Benoit Salomon**

INSERM / University of Toulouse, France

**Assoc Prof. Tan Meng How**

Nanyang Technological University, Singapore

**Prof. Elisa Laurenti**

University of Cambridge, United Kingdom

**Prof. Leon Schulte**

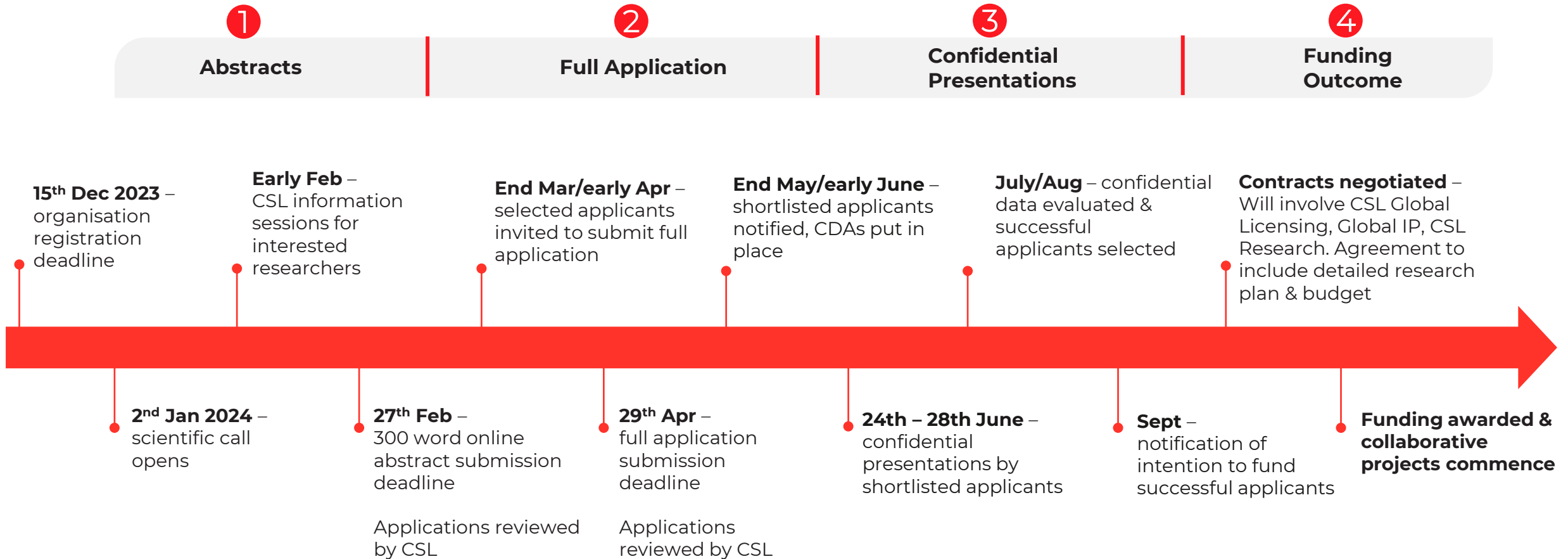
Philipps-Universität Marburg, Germany



Driven by **Our Promise**

# CSL

# CSL 2024 Research Acceleration Initiative Process



*No obligation for registered organizations to submit applications*

*No limitation on number of abstracts each registered organization can submit*

# Agreement Guidance



Separate collaboration agreements will be negotiated for each project which reflect the nature of the project, nature of funding and support, and the contributions of both parties



Under these negotiated agreements, CSL will be granted certain rights of interest to the program results for further R&D and/or commercialization



Collaboration agreements will typically include the following terms (although CSL may propose other conditions depending on the nature of the project):

- *Research organization will generally own results arising under the project*
  - CSL would typically own any results which relate to proprietary CSL products or materials contributed to the project or may seek joint-ownership of results to which it has made a significant contribution (e.g. protein or antibody discovery and engineering activities).
  - The RAI is designed to accelerate the translation of novel discoveries made by research scientists – for proposals outside this scope, we may propose that projects be progressed outside the RAI
- *CSL will be granted an exclusive option to negotiate an exclusive, worldwide licence*
- *CSL supports publication of research outcomes*

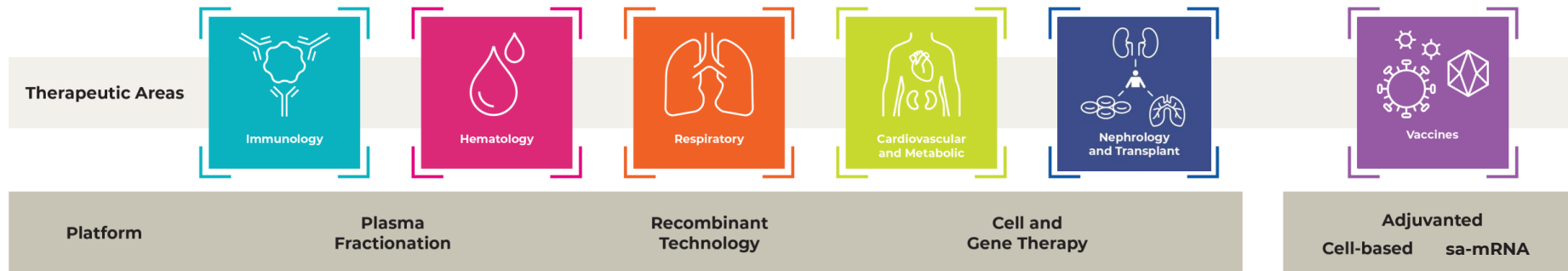


Further details on agreement terms can be provided on request

# Eligibility

To be eligible to apply, researchers/clinicians must satisfy the following 2 conditions:

1. Be employed by a research organization registered to participate in the 2024 Research Acceleration Initiative
2. Submit a 300-word online abstract that is aligned with CSL's Therapeutic Areas and scientific Platforms:



*Specific indications of focus within each TA are provided on slides 28-35.*



# Abstract submission via online portal

## Step 1/2 - Lead Investigator Information

Applications for the 2023 CSL Research Acceleration Initiative open 3rd January 2023 and close 23rd February 2023. Applications received outside these dates (before or after) will not be reviewed.

**Fields with \* are mandatory**

First Name \*

Last Name \*

Organization \*

Email \*

Address

City

Country \*

Salutation

Job Title \*

Phone

Confirm Email \*

Zip/Postcode

Geographical region \*

Are you an existing collaborator, or have you previously collaborated with CSL (including CSL Behring, CSL Seqirus or CSL Vifor)?

Yes  No

**CONTINUE**

## Step 2/2 - Describe your opportunity and confirm submission

Please describe and categorize your opportunity.

**Fields with \* are mandatory**

Proposal Title \*

Primary Therapeutic Area \*

Cardiovascular and Metabolic	Hematology	Immunology	Respiratory	Nephrology and Transplant	Vaccines	Not specific to a Therapeutic Area (e.g. platform technology)
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Secondary Therapeutic Area

Cardiovascular and Metabolic	Hematology	Immunology	Respiratory	Nephrology and Transplant	Vaccines	Not specific to a Therapeutic Area (e.g. platform technology)
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Indications \*

Modality \*

Cell therapy	Extracellular vesicles	Gene therapy
Oligonucleotide (siRNA, asRNA, miRNA)	Peptide	Plasma
Recombinant (incl. antibodies)	Small molecule	Other modality

Opportunity Type \*

Biomarker	New use for CSL product or pipeline candidate	Novel target or therapeutic candidate
Research Tool	Target Discovery	
Vaccines - mRNA/lipid nanoparticle platform improvements	Vaccines - influenza virus antigen purity/yield enhancements	
Vaccines - utilizing MF5009 adjuvant	Other	

Project Description (max. 300 words) \*

Example of what to include in Project Description: "We have discovered a novel target expressed on X cells. We have generated data in X assay(s) and/or X model(s). We have shown the mechanism of action in model(s) via X pathway(s). Inhibition of this target could be used to treat X indication(s). This novel strategy could address an important unmet need for patients and be superior to standard of care and other therapeutics in development for reasons X, X and X."

I have read the privacy policy and agree with it. [Read more...](#)

I hereby confirm that my submission does not contain any confidential information.

I'm not a robot

**BACK** **SUBMIT**

# What is involved for participating research organizations?



## Abstracts

- Internal promotion of initiative (*CSL to provide flyer*)
- Promotion of CSL information sessions/webinars for interested researchers
- Provide abstract submission portal link to researchers
- Discuss proposals with interested scientists ahead of 300-word abstract submission deadline



## Full Application

- Support shortlisted researchers with preparation of full proposal via CSL RAI application form
- Ensure no disclosure of confidential information prior to submission of applications to CSL



## Confidential Presentations

- Facilitation of CDA
- Assist with scheduling of confidential presentations to CSL



## Funding Outcome

- Contract negotiation
- Preparation of detailed research plan and budget in partnership with CSL

Connect CSL with the appropriate internal contact(s) for each stage of the process

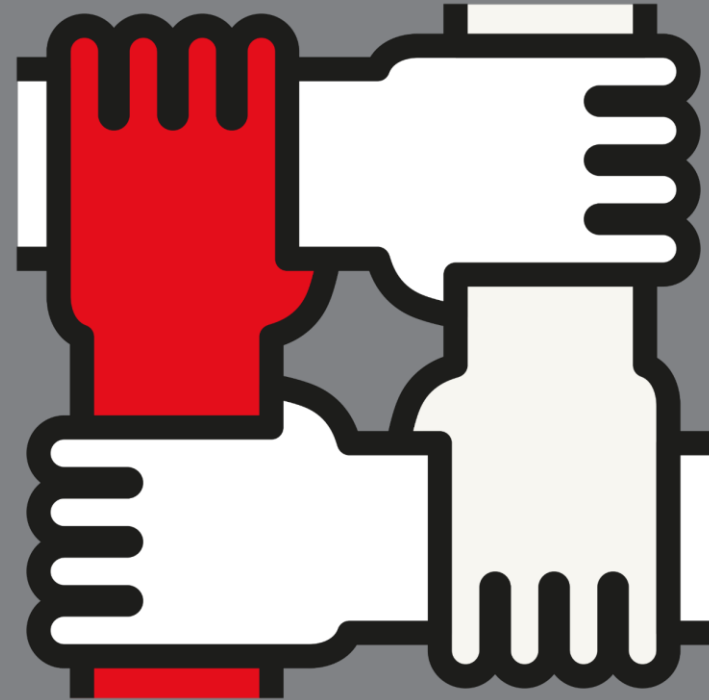
# What makes for a competitive proposal?

- ✓ Aligned with our focus areas and modalities (slides 28-35)
- ✓ Project is clearly defined (as opposed to a general overview of the applicant's research interests)
- ✓ Focused on a novel target or therapeutic candidate
- ✓ Clear differentiation of approach from competitors and current standard of care
- ✓ Research team has capacity and expertise to complete the bulk of the experimental work over the course of the program (with CSL guidance and support)
- ✓ If third party IP is required, ensure your research organization has secured all necessary rights to grant CSL an exclusive option to negotiate an exclusive, worldwide licence

# Examples of activities funded in previous RAI partnerships

- ✓ Human target validation and translational studies using patient samples
- ✓ Mechanism of action studies for therapeutic candidates
- ✓ Benchmarking to provide proof-of-concept for the differentiation of novel therapeutics to standard-of-care or competing therapeutics in development
- ✓ Target validation using genetic knock-out/knock-in or tool compounds in preclinical disease models
- ✓ Characterization of therapeutic candidates (e.g. affinity, potency, selectivity, and developability)

# Benefits of collaborating with CSL



# Benefits of CSL's Research Acceleration Initiative



**Collaborate**  
with one of the world's  
leading biotech  
companies



**Publish with CSL**  
200+ publications  
with our collaborators  
since 2020



**Funding**  
of up to  
\$400,000 USD  
over 2 years



**Access expertise**  
CSL scientific champion  
assigned to provide you  
with industry guidance



**Recognition**  
Awardees may use the title  
"CSL Research Acceleration  
Initiative Fellow"



**Accelerate**  
the translation of  
your research into  
new therapies

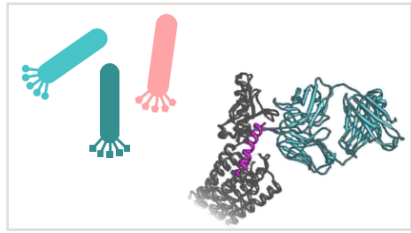


**Access global capabilities**  
in R&D, clinical, intellectual  
property, manufacturing  
and commercial

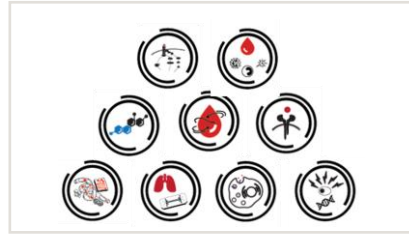


**Demonstrate impact**  
of your research to  
funding bodies via  
industry collaboration

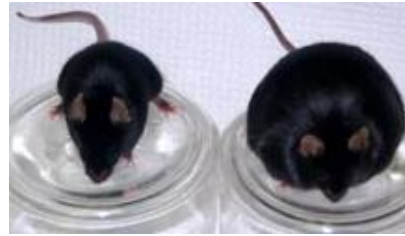
# Capabilities from Discovery to Patients



Antibody Discovery and Protein Engineering



In vitro pharmacology



Animal Models of Disease



Toxicology & Product Development



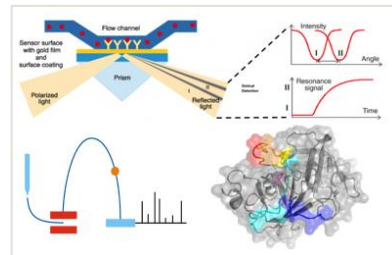
Patients

## R&D CAPABILITIES

## CLINICAL CAPABILITIES



Protein production and purification



Analytical Biochemistry

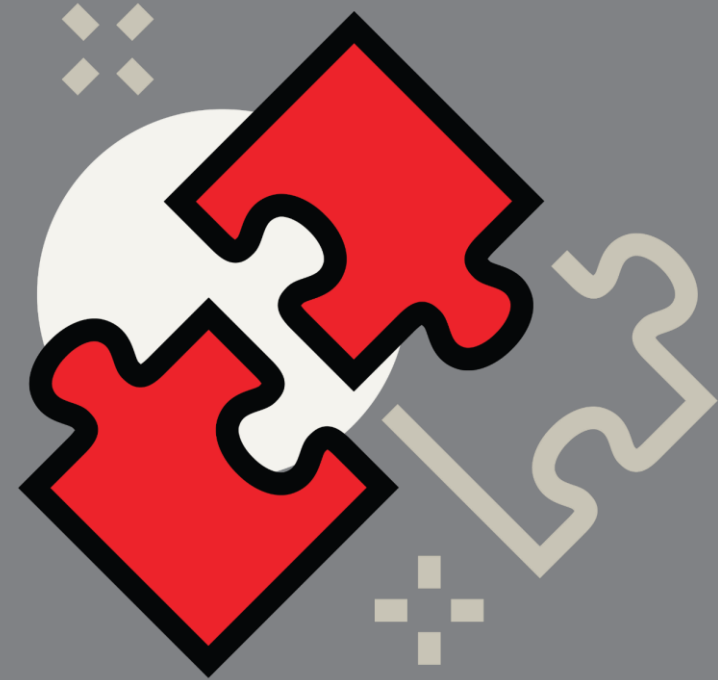


Translational Medicine & Data Science



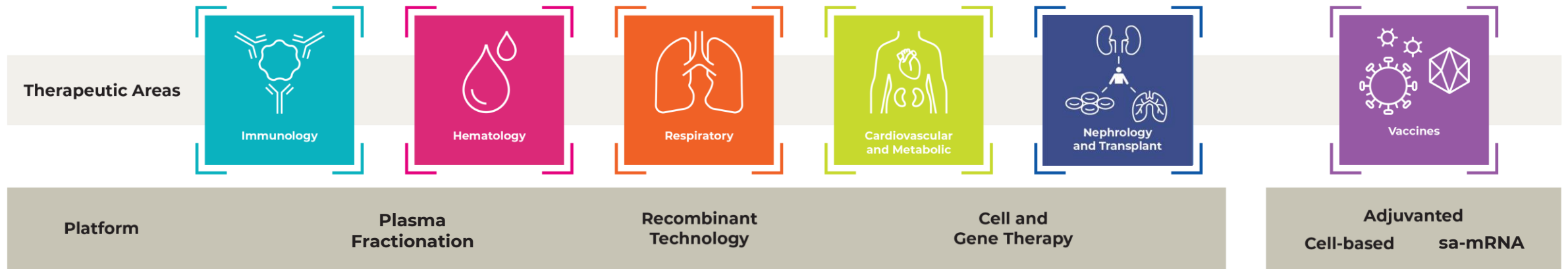
Phase I-III/Launch Manufacturing

# CSL's core Therapeutic Areas

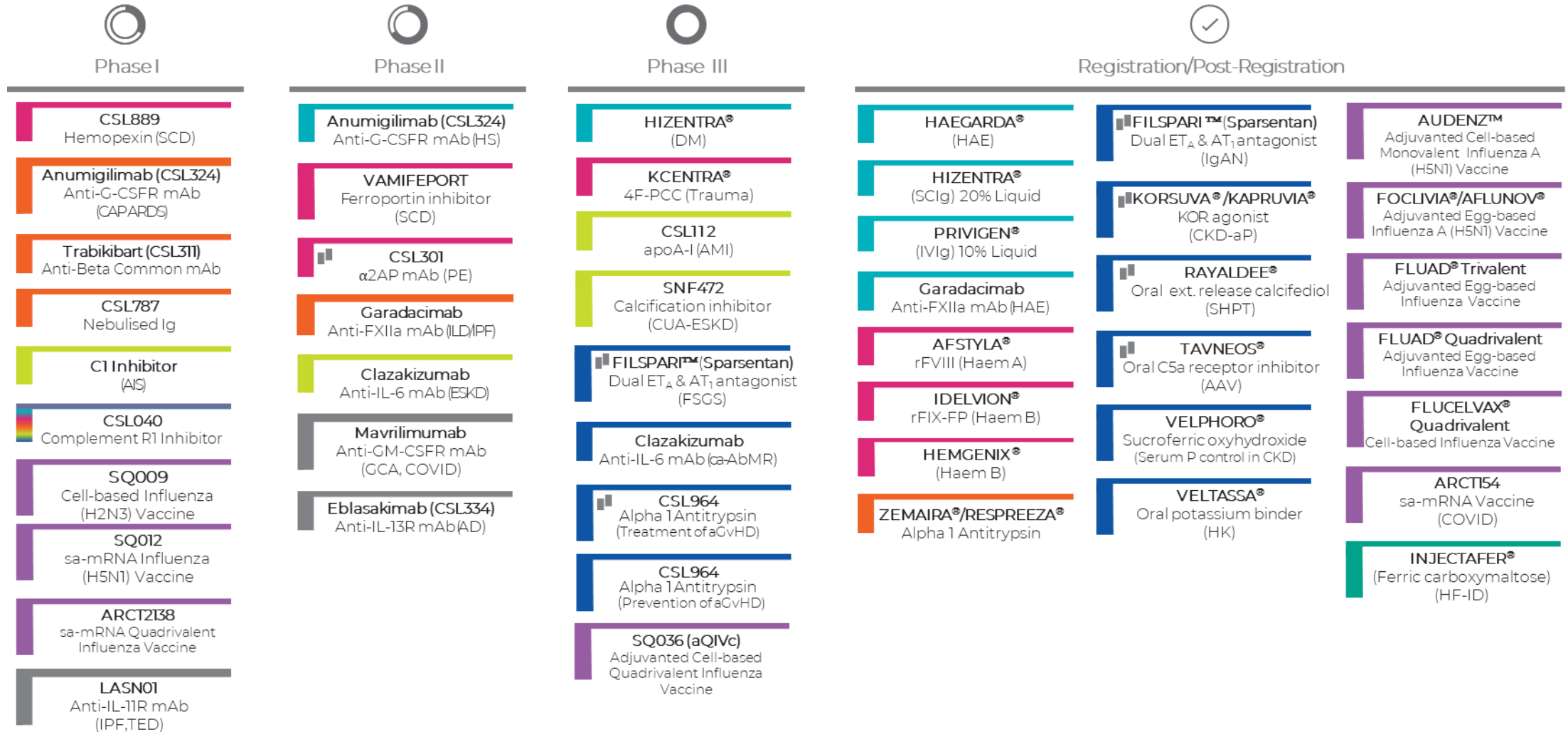




# CSL's Core Therapeutic Areas & Platforms



# CSL R&D Portfolio – FY24



■ Immunology     ■ Haematology     ■ Respiratory     ■ Cardiovascular & Metabolic     ■ Nephrology & Transplant  
■ Vaccines     ■ CSL Vifor     ■ Outlicensed Programs     ■ Partnered Projects

# Product and pipeline highlights



**Privigen®** (10% intravenous Ig)  
Primary immunodeficiencies (PID), Secondary Immune Deficiency (SID)\*, Chronic inflammatory demyelinating polyneuropathy (CIDP)

**Hizentra®** (20% subcutaneous Ig)  
PID, CIDP, SID\*  
Dermatomyositis (DM), Ph III

**Haegarda®** (C1 Esterase Inhibitor)  
Hereditary angioedema

**Garadacimab** (Anti-FXIIa mAb)  
Hereditary angioedema, Ph III

**CSL324** (Anti-G-CSFR mAb)  
Hidradenitis suppurativa (HS), Ph I



**Idelvion®** (Recombinant FIX-FP)  
Hemophilia B

**Hemgenix®** (AAV FIX gene therapy)  
Hemophilia B

**Afstyla®** (Recombinant FVIII)  
Hemophilia A

**Kcentra®** (Prothrombin complex concentrate)  
Urgent warfarin reversal

**Vamifeport** (Oral ferroportin inhibitor)  
Sickle cell disease, Ph IIa

**CSL889** (Hemopexin)  
Sickle cell disease, Ph I

**CSL888** (Haptoglobin)  
Sub-arachnoid hemorrhage, preclinical development

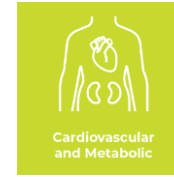


**ZEMAIRA®/RESPREEZA®**  
(Alpha 1 Antitrypsin)

**Garadacimab** (Anti-FXIIa mAb)  
Idiopathic Pulmonary Fibrosis, Ph IIa

**CSL311** (Anti-β-common mAb)  
Airways inflammation, Ph I

**CSL787** (Nebulized Ig)  
Respiratory infections, Ph I



**CSL112** (ApoA-1)  
Acute coronary syndrome, Ph III

**CSL300** (Anti-IL-6 mAb)  
End stage kidney disease Ph IIb



**FLUAD Quadrivalent**  
Adjuvanted Influenza Vaccine

**FLUCELVAX Quadrivalent**  
Cell-based Influenza Vaccine

**Adjuvanted Cell Culture Influenza Vaccine** (aQIVc), Ph II

**sa-mRNA Influenza Vaccine**, PC

**ARCT-154 COVID-19 Vaccine**, Ph III



**CSL964** (Alpha 1 Antitrypsin)  
Graft versus host disease, Ph III

**Clazakizumab** (Anti-IL-6 mAb)  
Antibody mediated rejection, Ph III

**CSL040** (Novel Complement Inhibitor), Ph I

\*ex-USA

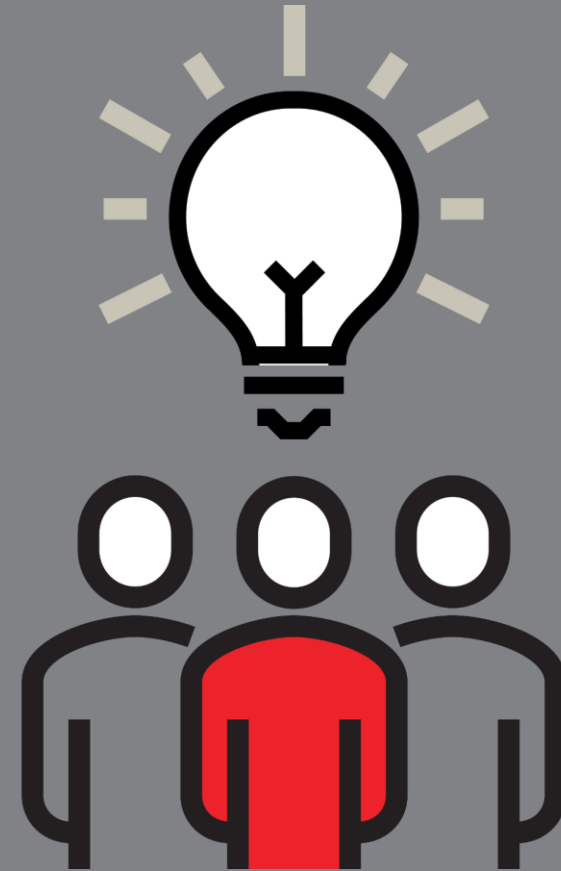
Our full pipeline can be viewed [here](#)

Driven by **Our Promise**

# Areas of interest for collaboration

## Areas not of interest

- *Oncology (including hematological malignancies)*
- *Medical devices or diagnostics*
- *Small molecule approaches*





## Immunology



## Core interests for early stage partnering

### Novel targets or best-in-class biologic therapeutics addressing:

1. B cell and plasma cell depletion or inhibition
2. T cell modulation, immune checkpoint agonism or co-stimulatory antagonism, regulatory T cell stimulation or tolerance
3. Modulation of cytokines, chemokines and immune-super family members (e.g., TNF, IL-1, other) , particularly approaches enabling multi-pathway inhibition
4. Depletion/modulation of innate immune effector cells

### Autoimmune diseases:

Inflammatory Idiopathic Myopathies including Dermatomyositis, Primary Sjögren's Syndrome, Pemphigus Vulgaris, Bullous Pemphigoid, Small Fiber Neuropathy, ANCA-Associated Vasculitis and Autoimmune Hepatitis

### Not of interest:

Target discovery campaigns or platforms, intracellular targets, complement inhibition



## Hematology



## Core interests for early stage partnering

### Acute hemorrhage control and hemorrhagic stroke

1. Novel biologic therapies to treat and prevent acute hemorrhage (e.g. intracerebral hemorrhage (ICH), reversal of anti-coagulation/anti-platelet associated bleeding)
2. Novel biologic targets and therapies for the treatment of secondary brain injury in subarachnoid hemorrhage and ICH
3. Omics approaches for patient stratification and drug discovery

### Acute thrombotic conditions (macro- and micro-circulation)

1. Novel biologic therapies for targeted fibrinolysis/thrombolysis in acute thrombosis (ischemic stroke, pulmonary embolism)
2. Novel biologic therapies to treat and prevent microvascular thrombosis and endotheliopathies (e.g. TMAs, APS and DIC).

### Benign hematology adjacencies\*

1. Novel biologic therapies for the treatment of anemias
2. Novel biologic therapies to treat bone marrow disorders



## Respiratory



## Core interests for early stage partnering

### **Idiopathic pulmonary fibrosis (IPF), pulmonary sarcoidosis and progressive pulmonary fibrosis (PPF)**

1. Novel biologic therapies or target proposals derived from translational or biobank cohorts
2. Therapies targeted at reversing remodelling of fibrotic lung tissue
3. Multiomics-based approaches to target discovery

### **Community acquired pneumonia (CAP)-associated complications**

(Acute Respiratory Distress Syndrome (ARDS), Sepsis, Acute kidney injury)

1. Novel biologic therapies or target proposals derived from translational or biobank cohorts
2. In Silico approaches for patient stratification to delineate CAP patients at risk for ARDS/Sepsis/AKI



## Cardiovascular and Metabolic



# Core interests for early stage partnering

### Major Adverse Cardiovascular Event (MACE) prevention

Atherosclerotic plaque stabilization in severe disease

### Rare lipid disorders

Novel targets or biologic therapies (including gene therapies) for rare lipid disorders e.g. homozygous familial hypercholesterolemia

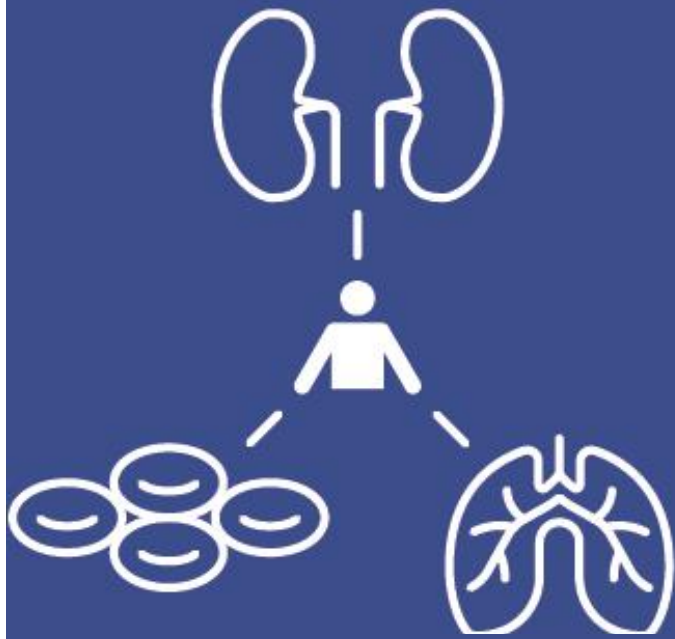
### Myocarditis

Novel targets or biologic therapies for myocarditis  
Biomarker approaches for patient stratification

### Inflammatory cardiomyopathies

Novel targets or biologic therapies for inflammatory cardiomyopathies





# Nephrology and Transplant



## Core interests for early stage partnering

### **Acute and chronic solid organ transplant rejection (kidney/lung) therapies**

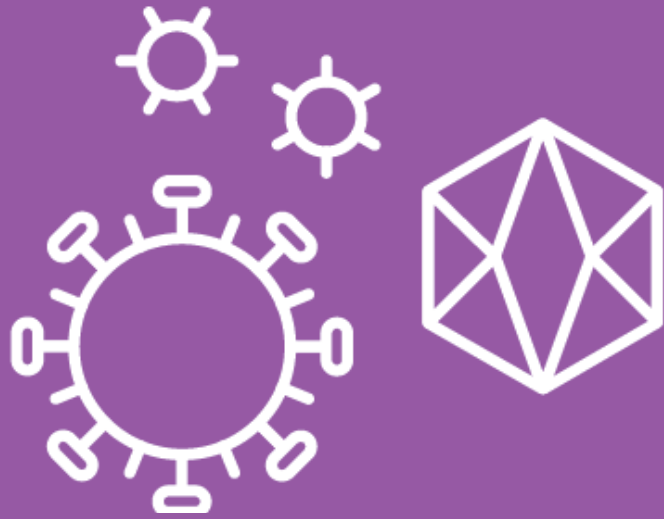
Novel biologic therapies or targets to prevent or treat acute and chronic solid organ transplant rejection of the kidney and lung

### **Chronic graft versus host disease (GvHD)**

Novel biologic therapies for the treatment and prevention of chronic GvHD

### **Tolerance for organ transplant rejection**

Novel biologic therapies for the induction of tolerance to prevent or treat organ transplant rejection



# Vaccines



## Core interests for early stage partnering

### Respiratory vaccines

1. New antigenic targets (epitopes or combinations)
2. Methods (e.g. AI/machine learning) to predict respiratory viral evolution/pathogenicity to inform vaccine development

### New vaccine targets

Development of novel targets/approaches for any disease

### RNA delivery and therapeutics

1. RNA delivery, enhanced stability, route of administration and/or expression strategies
2. mRNA-encoded protein therapies encompassing cellular targeting technologies

### Immune mechanisms

Understanding innate and adaptive responses to vaccines



## Cell & Gene Therapy



# Core interests for early stage partnering

## Gene editing / genomics

1. Improve insertional editing efficiencies *in vivo*
2. Genetic elements enhancing regulation of cells of the immune system (e.g. promoters and enhancers)

## In vivo Delivery

1. Delivering nucleic acid templates for insertional gene editing
2. Targeting moiety for HSCs

## GT safety

Technologies that minimize SAEs from insertional gene editing

## Areas not of interest

- Oncology (including hematological malignancies)
- Ex vivo cell therapy



## Plasma Protein Research



## Core interests for early stage partnering

### Novel plasma therapeutic candidates

1. All diseases considered. Candidates aligned with CSL's therapeutic areas will be prioritized
2. CSL can provide native human plasma proteins ( $\geq \mu\text{g/L}$  plasma concentration) for preclinical proof-of-concept studies







### Novel association of plasma protein function with disease

1. Based on healthy and patient clinical data sets, or
2. Access to patient data sets with corresponding clinical data to enable association studies to be performed

### Novel methods for plasma protein purification

Protein purification systems capable of targeted purification from plasma with high purity at research scale (methods translatable to manufacturing scale will be prioritized)

# Checklist for 2024 Research Acceleration Initiative

-  Register your research organization by 15<sup>th</sup> Dec 2023 by emailing [RAI@csl.com.au](mailto:RAI@csl.com.au) (NB: 2023 participants are automatically re-enrolled)
-  Update your organization's primary contact details if required
-  CSL will provide flyer with primary contact details for promotion within your research organizations
-  CSL will provide the link to the online abstract submission portal
-  CSL will provide you with invitations to information webinars to share with interested researchers – webinars will be held in Feb 2024
-  Online portal opens for abstract submissions by researchers on 2<sup>nd</sup> Jan 2024 and closes on 27<sup>th</sup> Feb 2024



# Questions



# THANK YOU

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*Director,  
Research Innovation Europe*

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Dr Marco Cavallari  
*Senior Manager, Research  
Innovation Europe*

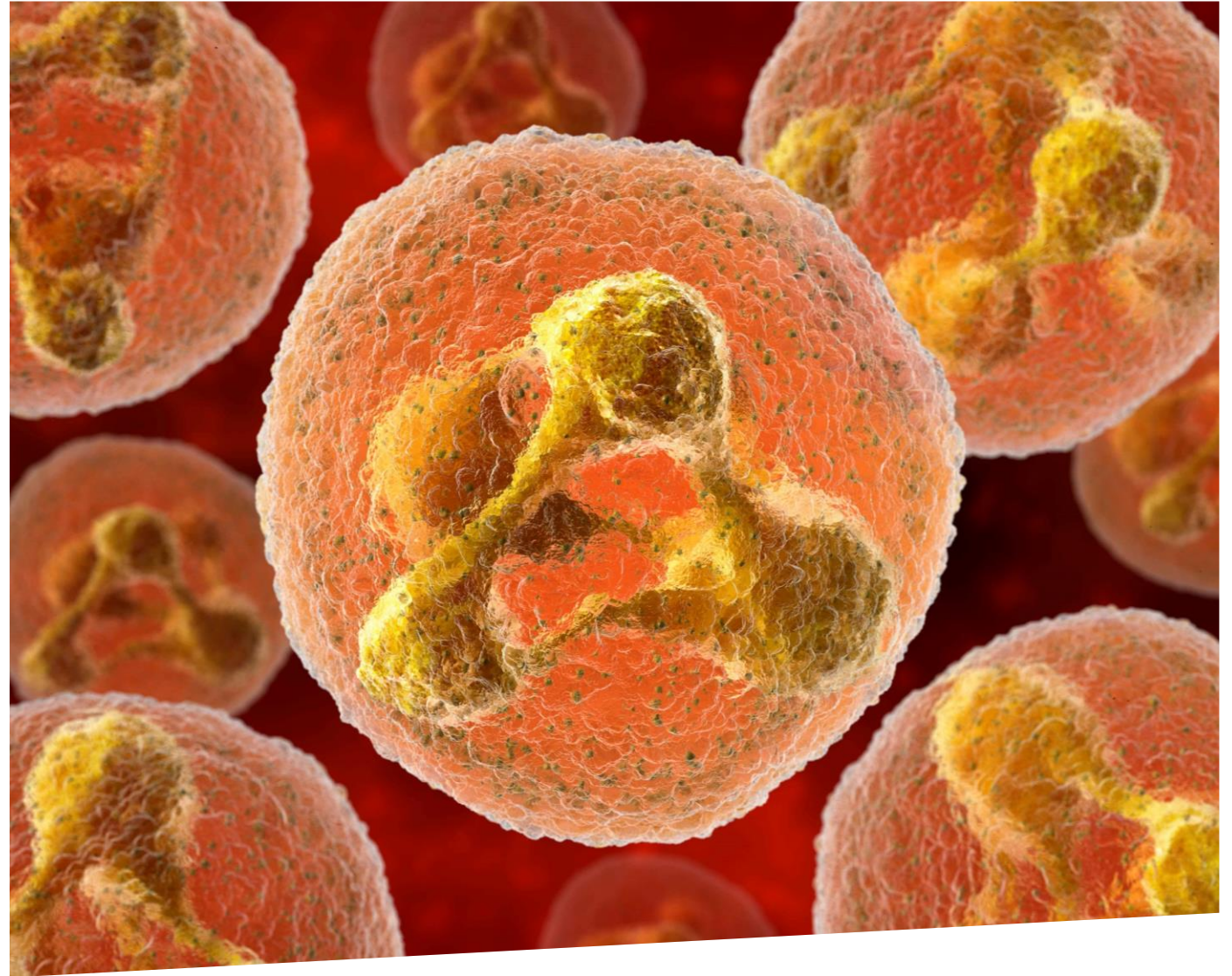
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[csl.com/csl-rai](http://csl.com/csl-rai)



# Terms and Conditions for Research Acceleration Initiative Portal ("RAI Portal")

1. This RAI Portal is an online portal operated by CSL Innovation Pty Ltd ("CSL") for the purpose of allowing individuals to submit scientific proposals for consideration by CSL for its Research Acceleration Initiative program. By using this website and the RAI Portal, and by providing your submission and personal information to CSL, you are agreeing to abide by these terms and conditions.
2. You acknowledge and agree that CSL has no obligations of confidentiality or non-use in relation to the submission provided. You warrant that your submission does not contain confidential information of any kind. Further, you acknowledge that notwithstanding the existence of any confidentiality agreements previously entered into between you and CSL, the terms of such agreements will not apply with respect to any information submitted by you through the RAI Portal.
3. You further represent and warrant that:
  - a. you have the right and authorization (including where relevant after consultation with all relevant commercialization or technology transfer offices) to submit an application to the RAI Portal and to accept the terms and conditions set out herein;
  - b. you are an employee or are otherwise affiliated with a registered organization authorized by CSL to submit an application to the RAI Portal; and
  - c. to the best of your knowledge and without making any further enquiries, the information provided in your submission (and CSL's use of that information in connection with the Research Acceleration Initiative program) shall not infringe on the intellectual property rights of any third party, including your current or former employer, university, public research institute or other registered organization.
4. CSL may disclose personal information collected in connection with your use of this website or the RAI Portal to your employer, university, public research institute or other registered organization (if applicable) as at the time your application was submitted, solely for the purpose of reviewing and determining your application. CSL will ensure that any personal information collected, used or disclosed in connection with your use of this website or the RAI Portal is handled in accordance with all relevant privacy legislation and with CSL's privacy policy, a copy of which is available at <https://www.csl.com/privacy-policy>.
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