



# The 2022 International workshop on Alport Syndrome

**In person in Calgary, Canada and online**

**7 September 2022  
8am-6pm**



**Event sponsor:**



**Detailed agenda  
and list of moderators  
and speakers**

**Education sponsors:**



**Patient sponsors:**



**sanofi**

**Questions to:**

[info@ipna22.org](mailto:info@ipna22.org) or [speakers@ipna22.org](mailto:speakers@ipna22.org)

**If either of these do not provide an answer, please contact Jiří Handl: [Jiri.Handl@c-in.eu](mailto:Jiri.Handl@c-in.eu)**

**T: +420 261 174 301 | M: +420 732 101 095**

**If you are still unable to contact anyone, then please email Alice Cooper**

**[workshops@alportsyndromealliance.org](mailto:workshops@alportsyndromealliance.org)**



**Alport Syndrome Alliance**  
A global network advancing treatments and knowledge

The activities of the Alport Syndrome Alliance are delivered by Alport UK, until the alliance is fully operational and sustainable.



# Welcome

## **Welcome to this workshop - the sixth in a series of immersive conversations - for an expanding Alport community**

Alport syndrome (AS) was named after Cecil A. Alport, who published his observations in the British Medical Journal in 1927 connecting kidney, hearing and eye issues, and later described in his book 'On Nephritis' in 1929. Sam Clarke's inspiring short video #fightingfailure (<https://youtu.be/1A1adCj-cvo>) captures the rollercoaster journey of living with an isolating, rare, inherited condition - Alport Syndrome - that impacts many members of a family.

Groups of international researchers working on Alport in the 1960s, 70s, 80s and 90s (1990s work funded by the European Commission) demonstrated the power of collaboration with their work to progress the understanding of Alport Syndrome. National patient organisations emerged, set up scientific advisory groups and raised vital funds for research in USA (Alport Syndrome Foundation), in France (AIRG France), and Italy (ASAL Onlus). Initially there were not enough research projects applying for funding. The inspiration, valuable publications and vital outputs from the various research and patient collaborations inspired this workshop series. This online workshop is the sixth workshop in a series of informal, immersive workshops. The previous workshops were 'in-person': Oxford, UK (2013), Göttingen, Germany (2015), Glasgow, UK (2017), Siena, Italy (2019) and online (2021). Highlights from the last in person workshop - The 2019 International workshop on Alport Syndrome – in Siena University, October 2019, can be viewed at <https://youtu.be/QH8mDTmKaVU>. In between workshops, a number of working groups are emerging on topics on which we want to collaborate: Basic science, Genetic variants, Patient registries, Naming, Clinical and clinical trials. Each is in different stages of development and will share updates at this workshop.

### **Despite the pandemic, more people engaged**

The fifth workshop was supposed to be in Beijing, China, but was necessarily postponed because of Covid. However, the pandemic was an opportunity for the community to connect online instead, and engage a wider, more diverse community. With the inspiration and leadership from the Lennon and Miner labs, and a host of patient volunteers, a further series of 26 Alport online workshops featured the latest research and labs all over the world. These online workshops engaged a wider and more diverse group of people during the various country lockdowns, moderated by young adults living with Alport Syndrome. What was so inspiring was that many of the Alport volunteers in each country personally worked on the frontline during the pandemic and kept people safe.

## **Volunteering and results of the contributions**

Everyone who participates in these workshops is a volunteer and contributes their own time, on top of student studies, 'day' jobs and busy lives. The people living with Alport Syndrome are incredibly grateful to everyone who contributes this time, energy, and inspiration. We welcome new volunteers to the community to contribute on all aspects of the work, to represent their country, and to enable connections with an even wider international community. Please email us: [workshops@alportsyndromealliance.org](mailto:workshops@alportsyndromealliance.org).

We should all feel very proud of the outcomes from this vibrant collaborative community – the numbers speak for themselves:

- **First workshop in 2014 in Oxford, UK:** 75 participants from 17 countries with 34 abstracts, but no pharmaceutical or commercial companies involved
- **Fourth workshop in 2019 in Siena, Italy:** 146 participants, including 36 patients and representatives from 12 pharmaceutical companies, plus 46 posters. A survey of committee members and teams celebrated: 131 publications, 16 book chapters and 38 grants – over 90% were collaborative efforts
- **Fifth workshop online in 2021:** A three-day workshop with over 220 registrations from 26 countries, of which a third were patients, and featuring 40 posters and over 60 people contributing to the workshop, either as presenters or moderators.
- **This sixth workshop in 2022: online and in Calgary, Canada,** as a one-day pre-workshop to the 19<sup>th</sup> congress of the International Paediatric Nephrology Association (IPNA). We have some interesting research on hearing, which we hope will advance our understanding of Alport Syndrome, along with updates on the latest clinical trials. We will not have time to feature as much of the latest basic science research this time, covering only highlights.

Many pharmaceutical representatives now participate in these workshops, representing a range of companies advancing treatments, doing pre-clinical studies, or planning/delivering clinical trials.

## **What are the important ingredients in this community that make it work?**

- the strong leaders – each experts in their own right – that collaborate and find ways to work together internationally, sharing ideas and resources, avoiding unnecessary ego and hierarchy (you will note we do not use titles) in our drive to advance treatments and knowledge that creates a brighter future for people living with Alport Syndrome
- the mix of diverse perspectives, volunteering and offering to contribute at many different levels, means we look at the challenges and opportunities from different angles. The different perspectives come from patients or patient families, clinicians, pathologists or laboratory scientists (genetics, kidneys, hearing, eyes), pharmaceutical or commercial company representatives, epidemiologists etc plus early career people, through to those with more experience
- the personal choice, drive and motivation of individual leaders and teams to engage, volunteer or scope projects, get funding and deliver research, to build knowledge and advance treatments
- the sense of 'extended family' or 'virtual collaborative' structure, which bonds us together, means we watch out for each other, our families, our lives, our careers
- Even funding these workshops is a collaborative effort; the workshop this year is funded by fundraising in the UK and international sponsorship. We are very grateful this year to the following organisations for sponsoring The 2022 International workshop on Alport Syndrome: Alport UK, Travers Therapeutics, Eloxx Pharmaceuticals, Kidney Foundation of Canada and Sanofi.

### **How to get the most out of the workshop**

Those who participated in our four 'in-person' workshops will know that the 'immersive' element of the workshop was because we 'locked the doors', everyone sat in a circle and contributed to the workshop discussions and **asked questions**. The more you contribute, the more you get out of it. You will get the most out of this workshop if you actively participate in the discussion, ask questions either in the room or online.

### **Topics to cover in 2023 workshop**

The exciting developments in basic science are one of the missing elements from this workshop and there will be other topics missing too. We are not trying to cover everything today. This one-day workshop is designed as part of the 19<sup>th</sup> World Congress for IPNA to capture the attention of the children's kidney doctors participating in the wider IPNA agenda. Let us know what topics are missing and we will design more specific sessions for the 2023 workshop on these topics to give them the focus they need. We will carry on the discussions after the workshop too, aiming to produce publications and further define what the Alport Syndrome Alliance needs to work on to continue the process for this global network to advance treatments and knowledge.

### **Nominations for The 2022 Cecil Alport Awards**

This year, we will celebrate patient contributions to the workshop, whether this be questions, videos or moderation. Email your nominations for patient award winners for The 2022 Cecil Alport Awards to [workshops@alportsyndromealliance.org](mailto:workshops@alportsyndromealliance.org) by 1800 hours Calgary time (0100 BST).

We look forward to the conversations. Thank you for participating, especially as it is a busy time as we all work out how to work with the implications of the pandemic. Enjoy this workshop as we shine a light on Alport research.

Susie Gear, Julian Midgeley


On behalf of the Workshop Organising Committee

**The 2022 online International workshop on Alport Syndrome**

## **The 2022 Cecil Alport Awards**

Recognising achievement in researching Alport Syndrome

- 1. The best contribution from a patient participating for the first time**
- 2. The best young patient contributor**
- 3. The best patient question asked at The 2022 International workshop on Alport syndrome**
- 4. The best patient moderator**



## Workshop objectives and design principles for the workshop

### Workshop objectives

- **Spotlight the latest developments in Alport Syndrome - connect and update the international clinical community on patient stories, diagnosis and clinical guidelines, naming, hearing, registries, and clinical trials**
- **Connect the Canadian patients (in person) with the wider international Alport community of patients, clinicians and research scientists and bring young adult patients (aged 18-35) together internationally**
- **Be part of the future of Alport Syndrome research and treatment.**

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### Principles for the design of the workshop

- We encourage you to join as many sessions as you can.
- As the audience is made up of a third who potentially have non-science backgrounds (patients), and there is a huge range of expertise represented that may not have deep knowledge in a particular area of the speaker's science, please can all speakers explain the terms they use and avoid abbreviations
- The workshop is run as a 'discussion' format, so participants are actively encouraged to make comments and ask questions, either in person or online.
- It is an International '**workshop**', so speaking slots are limited to allow as much time for **dialogue and discussion**.
- We very much welcome questions and participation; participants have a key role to encourage questions and deeper debate. There is even a Cecil Alport Award for the best question at the workshop.

## **Social Media Policy**

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### **Purpose:**

- We are keen to have a social media 'buzz' around the meeting to help get other patients and researchers interested.
- Maintain confidentiality of research data, while also sharing information about Alport syndrome advances with the broader scientific community and public.

1. Participants are encouraged to share their experience and photos on social media during the meeting using the hashtag #AlportWorkshop
2. Do not share photos or information about unpublished work without permission from the presenter
3. Do not share photos of patients without their consent
4. Do not share photos of slides or posters without the permission of the presenter

## **The 2022 Workshop Organising Committee**

**Dr Marina Aksenova**, Veltischev Research and Clinical Institute for Pediatrics of the Pirogov Russian National Research Medical University, Moscow, Russia

**Assistant Professor Moumita Barua**, Toronto General Hospital, Toronto, Canada

**Dr Agnė Čerkauskaitė**, Division of diagnosis and treatment of Rare Kidney and Metabolic Diseases, Nephrology Center, Vilnius University Hospital Santaros Klinikos, Vilnius, Lithuania

**Professor Constantinos Deltas**, University of Cyprus, Molecular Medicine Research Center, Nicosia, Cyprus

**Professor Jie Ding**, Department of Pediatrics, Peking University First Hospital, Beijing, China

**Professor Frances Flinter**, Department of Clinical Genetics, Guy's Hospital, London, UK

**Professor Daniel Gale**, Department of Renal Medicine, University College London, UK

**Professor Danica Galešić-Ljubanović**, Department of Pathology University of Zagreb School of Medicine, Head of Department of Nephropathology and Electron Microscopy, Dubrava University Hospital, Zagreb, Croatia

**Professor Oliver Gross**, Department of Nephrology and Rheumatology, Georg-August-Universität Göttingen, Germany

**Professor Julia Höfele**, Institute of Human Genetics, Munich, Germany

**Professor Hirofumi Kai**, Graduate School of Pharmaceutical Sciences, Kumamoto University, Japan

**Professor Clifford Kashtan**, Department of Pediatrics, University of Minnesota, USA

**Professor Bertrand Knebelmann**, Necker Hospital, Paris, France

**Associate Professor Ron Korstanje**, The Jackson Laboratory, Bar Harbor, USA

**Professor Rachel Lennon**, Wellcome Trust Centre for Cell-Matrix Research, University of Manchester, UK

**Associate Professor Beata Lipska**, Department of Biology and Medical Genetics, Medical University of Gdańsk

**Dr Laura Massella**, Dipartimento di Nefrologia e Urologia, Ospedale Pediatrico Bambino Gesù, Rome, Italy

**Associate Professor Julian Midgley**, Department of Paediatrics, Alberta Children's Hospital, Canada

**Professor Jeffrey Miner**, Washington University in St. Louis, USA

**Professor Laura Perin**, Saban Research Institute, University of Southern California, USA

**Professor Judith Savige**, University of Melbourne, Australia

**Professor Alessandra Renieri**, Medical Genetics Unit, Azienda Ospedaliera Universitaria Senese viale Bracci 2, Siena, Italy

**Associate Professor Michelle Rheault**, Department of Pediatrics, University of Minnesota, USA

**Professor Roser Torra**, Fundació Puigvert, Barcelona, Spain

**Professor Neil Turner**, Department of Nephrology, Royal Infirmary of Edinburgh, UK

### **Representatives from the national patient organisations**

**Dave Blatt**, Alport Foundation of Australia

**Lisa Bonebrake and Andre Weinstock**, Alport Syndrome Foundation, USA

**Maria José Cacharron**, AIRG Spain

**Christof Finkler and Ute Rosenthal**, Alport Selbsthilfe, Germany

**Susie Gear, Tim McLean and Amanda McLean**, alport uk

**Daniela Lai and Rossella Ferrari**, A.S.A.L., Associazione Sindrome di Alport, Italy

**Julia Schifter**, Alport Foundation Israel

**Renée de Wildt**, Nierpatiënten Vereniging Nederland (NVN), The Netherlands

**Jessie Zhang and Mr Cai**, Chinese patient group, China

## **The Workshop Organising Committee principles for each topic**

to enable as much engagement as possible

<i>Duration</i>	<i>Type of contribution</i>	<i>Role and format</i>
5 mins	<b>Introduction</b>	Moderator
10-15 mins	<b>Presentations</b>	On topics
10 mins	<b>Q&amp;A and discussion</b>	Facilitated by the moderators



**alport**WARRIORS

[www.alportuk.org](http://www.alportuk.org)



**The Workshop Organising Committee  
would like to thank the many**

**alport**WARRIORS

**on Facebook for their openness to  
constructively help each other,  
suggest ideas for research and  
being the inspiration for**

**The 2022 International workshop on  
Alport Syndrome**



**a brighter future** for those living with alport syndrome



## Detailed agenda

**Please note all times listed are MDT – local Calgary time**

Start	Section	Topic	Speaker	Moderators
7.30	Registration			
8.00	<b>Welcome and Patient introduction</b>	Check in and Welcome <b>Living with Alport Syndrome: Joseph McLean</b> <b>Mental health: Don't Wait Fund</b>	Julian Midgley and Susie Gear	Aura Zealey Smith
8.30	<b>Keynote talk</b>	<b>Advancements and unmet needs in Alport syndrome</b>	Rachel Lennon (online)	Jeff Miner
9.00	<b>Basic science</b>	<b>Update: Alport animal models</b> <b>Update: In vitro research resources</b>	Jeff Miner Laura Perrin (online)	Constantinos Deltas (online) Rachel Lennon (online) Yanqin Zhang (online)
9.30	<b>Genetics</b>	<b>When to order a genetic test in general</b>  <b>What happens in the genetics lab – making sense of variants</b>	Judy Savige (online) Julia Höfele	Moumita Barua Frances Flinter (online), Constantinos Deltas (online)
10.00	<b>Diagnosis</b>	<b>Haematuria evolution in children to adult – presenting with urine infections</b>	Laura Massella (online)	Michelle Rheault Rachel Lennon (online)
10.30	BREAK			
10.50	<b>Video</b>	<b>Hall of hope: hope for the future</b>	Kimiya Bungler	
10.55	<b>Diagnosis</b>	<b>When to suspect Alport and order genetic testing or a kidney biopsy</b>  <b>FSGS as part of Alport spectrum disorders</b>	Roser Torra (online) Danica Galešić-Ljubanović (online)	Michelle Rheault Moumita Barua Rachel Lennon (online)
11.35	<b>Hearing</b>	<b>Hearing loss in Alport Syndrome – when to get hearing aids</b>	Stamatia Staikoudi (online)	Michelle Rheault Rachel Lennon (online)

12.00	<b>Hearing</b>	<b>Advancements in understanding the otopathology of Alport syndrome in humans</b>	Felipe Santos (online)	Lisa Bonebrake Jeff Miner Rachel Lennon (online)
12.30	<b>Hearing</b>		Marco Mandalà (online) Alessandra Renieri (online)	Jeff Miner Rachel Lennon (online)
13.00	LUNCH			
13.50	<b>Video</b>	<b>My Only Antidote:</b> trailer for a feature film	Sam Clarke	
13.55	<b>Alport Syndrome Alliance</b>	<b>Naming of Alport Syndrome</b> – project update	Alicia Byrne	Rachel Lennon (online) Roser Torra (online)
14.20	<b>Clinical guidelines</b>	<b>Clinical guidelines:</b> preventing progression	Neil Turner	Moumita Barua Michelle Rheault Agnė Čerkauskaitė (online) Fang Wang (online)
14.40	<b>Alport Syndrome Alliance</b>	<b>Alport Syndrome Alliance resource base/digital platform:</b> investing in the future	Sam Clarke	Ali Hariri (online) Neil Turner
14.50	<b>Alport Syndrome Alliance</b>	<b>Country updates:</b> <b>A.S.A.L Onlus – Italy</b>  <b>Nigeria</b>  <b>Alport UK</b>  <b>Alport Syndrome Foundation, USA</b>  <b>Alport Selbsthilfe</b>  <b>Croatia</b>	Rossella Ferrari, Italy Emmanuel Oduware, Nigeria Patrick Walker and Aura Zealey Smith, UK Lisa Bonebrake, USA Christof Finkler (online) Snježana Pavić (online tbc)	Agnė Čerkauskaitė (online)
15.40	BREAK			
16.00	<b>Research</b>	<b>Alport research hub: Update on opportunity for potential collaboration</b>	Rachel Lennon (online)	Aura Zealey-Smith Jeff Miner

16.10	<b>Alport Syndrome Alliance</b>	<b>Patient Registries: Current Status, Considerations for Data Collection, Value of Data</b>	Lisa Bonebrake Danny Gale (online) Ali Hariri	Neil Turner Agnė Čerkauskaitė (online) Marina Aksenova (online) Roser Torra (online) Bertrand Knebelmann (online)
16.40	<b>Keynote talk</b>	<b>New treatment options for Alport Syndrome</b> Overview of potential clinical trials	Oliver Gross (Recorded talk and in person for Q&A)	Bertrand Knebelmann (online) Michelle Rheault
17.30	<b>Summary</b>	<b>Summarise key takeaways for:</b> <ul style="list-style-type: none"> <li>• Clinicians</li> <li>• Laboratory scientists</li> <li>• Industry/commercial partners - Pharmaceutical and biotech companies</li> <li>• Patients</li> </ul>	Michelle Rheault Emmanuel Oduware Louise Hopkinson Rachel Lennon (online) Neil Turner Aura Zealey Smith Elia Galli Joy Toal	Susie Gear Roser Torra (online)
17.45		Check out	Mentimeter poll	Susie Gear
18.00	CLOSE			
19.00	Cultural tour	Walking tour of Calgary		
20.00	Dinner	Informal supper with Cecil Alport Awards for patient contributions		



The Workshop Organising Committee would like to thank Education sponsor

**TRAVERE Therapeutics**

for generously supporting

The 2022 International workshop on Alport Syndrome



The Workshop Organising Committee would like to thank Education sponsor

**Eloxx Pharmaceuticals**

for generously supporting

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The Workshop Organising Committee  
would like to thank patient sponsor

**Kidney Foundation of Canada**

for sponsoring the Patient participation at  
The 2022 online International workshop on Alport Syndrome



The Workshop Organising Committee  
would like to thank patient sponsor

**Sanofi Global**

for sponsoring the Patient participation at  
The 2022 online International workshop on Alport Syndrome

The programme for The 2022 International workshop on Alport Syndrome is a collaborative effort, put together by The 2022 Workshop Organising Committee.

Sanofi Global has provided sponsorship to this workshop but had no input into the agenda for this meeting, but as part of the sponsorship for this workshop, is allowed to participate in the workshop free of charge.



## The 2022 International workshop on Alport Syndrome

Itinerary for social activities – all participating in person are welcome to join

<b>Monday 5 September</b>			
6pm	Dinner	Casual dress	Milestones Restaurant <a href="https://milestonesrestaurants.com/locations/stephen-ave/">https://milestonesrestaurants.com/locations/stephen-ave/</a>
<b>Tuesday 6 September</b>			
12pm	Day for national patient groups and local Canadian/USA community to connect	Casual dress	Upper Select Boardroom in Calgary TELUS Convention Centre (North Building entrance)
1pm	Pizza lunch	Casual dress	<a href="http://www.pizzaitaliano.ca">www.pizzaitaliano.ca</a> 5 minutes' walk from Telus Convention Centre (walk-in) 2-140 11 Ave SW, Calgary, T2R 0B8
1pm-4pm	Planning	Casual dress	Richardson Wealth 525 8 Ave SW #4700 Calgary AB T2P 1G1 <a href="https://www.richardsonwealth.com">https://www.richardsonwealth.com</a>
3pm	Show round of the Telus convention centre by C-IN	Casual dress	Calgary TELUS Convention Centre (North Building entrance) Room Telus 101/102
6.30pm-10pm	Reception hosted by Liz and Kurt Pedersen	Casual dress	Pedersen home, 2605 Linden Drive SW, Calgary T3E 6C8
	Young adult activities following the reception include disc golf, pickleball, spike ball and a walkabout/sightseeing	Casual - sneakers recommended	
<b>Wednesday 7 September</b>			
7.30am	In person registration for The 2022 International workshop on Alport Syndrome	Casual dress	Calgary TELUS Convention Centre (North Building entrance)
8am	Event begins		
6pm	Event closes		

7pm	Walking tour. Taking in some of Calgary's most famous sights including the Calgary Tower, the Old Fire Station, Stephen Avenue, and several pieces of public art, all wound together with interesting stories of the city.	Casual - wear and comfortable shoes	Collecting from Marriott and Hyatt Regency hotels, finishing at dinner location (Charbar). <a href="https://calgarywbtours.com">https://calgarywbtours.com</a>
8.30pm	Dinner at Charbar	Casual dress	Charbar <a href="http://www.charbar.ca">www.charbar.ca</a>

<b>Thursday 8 to Sunday 11 September</b>			
8am	IPNA Main Congress Programme		<a href="https://www.ipna2022.org">https://www.ipna2022.org</a>



## Useful addresses

### 5 September @ 6pm

#### Milestones

107 8th Avenue SE

Calgary

T2G 0K4

<https://milestonesrestaurants.com/locations/stephen-ave/>

### 6-7 September

#### Calgary TELUS Convention Centre

North Building entrance

136 Eighth Avenue SE

Calgary, AB T2G 0K6

<https://calgary-convention.com>

#### Parking for Calgary Convention Centre

Convention Centre Parkade (Lot 60)

727 1st St SE

Calgary, AB T2G 0K7

### 6 September @ 1pm

#### Pizza Italiano

2-140 11 Ave SW

Calgary

AB T2R 0B8

Tel (403) 514-6000

<https://pizzaitaliano.ca>

#### Richardson Wealth

525 8 Ave SW #4700

Calgary

AB T2P 1G1

<https://www.richardsonwealth.com>

### 6 September @ 6.30-10pm

#### Kurt & Liz Pedersen

2605 Linden Drive SW

Calgary

T3E 6C8

### 7 September @ 8.30pm

#### Charbar

Simmons Building

618 Confluence Way SE

Calgary

AB T2G 0G1

<http://www.charbar.ca>

## **Accommodation**

### **Hyatt Regency Calgary** (walking tour pickup location)

700 Centre Street SE

Calgary

T2G 5P6

<https://bit.ly/3AtJGQI>

### **Calgary Marriott Downtown Hotel** (walking tour pickup location)

110 9 Ave SE

Calgary

T2G 5A6

<https://bit.ly/3zVIncQ>

### **Westin Calgary**

320 4th Avenue SW

Calgary

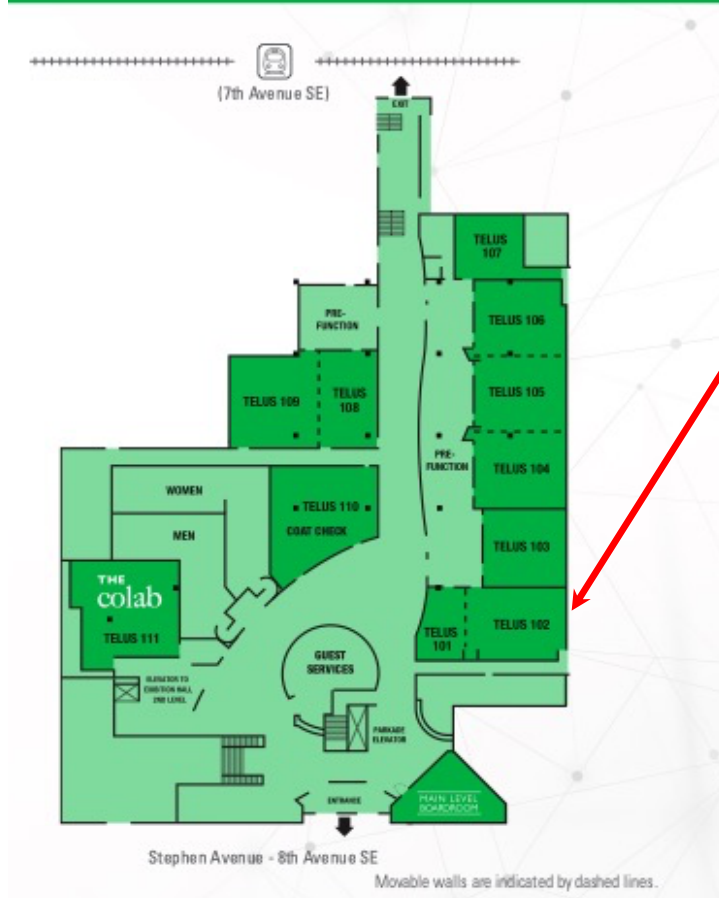
T2P 2S6

<https://bit.ly/3K25p59>

# The 2022 International workshop on Alport Syndrome

## Telus Convention Centre

- **Telus 101/102** allocated to The 2022 International workshop on Alport Syndrome. These are two rooms, which will be joined together.
- Capacity approx. 100
- Currently we have 67 in person with IPNA and Alport Syndrome Alliance registrations (24/8/22)



- Presentations will likely take place on one of the long walls
- Suggested layout as discussed in semi circles

