Aspire Biosciences

Systematic Serendipity for Rare Disease

Systematic Serendipity: Our methodology for developing new treatments for rare diseases

The Problem

7000+ rare diseases.

95% have no treatment

Small Market Barrier

Rare diseases are **not perceived as lucrative** for resource allocation

Low Awareness & Inadequate Information

Insufficient research and **lack of awareness** render search costs high

The Luck Factor

Serendipity often plays a key role in identifying rare disease treatments

An Example

Chance meeting

Patient Group founder presents a poster with **symptom list**, Pharma CEO has partially validated **molecule**

Idea

Unmet needs from both sides **align**

Clinical development

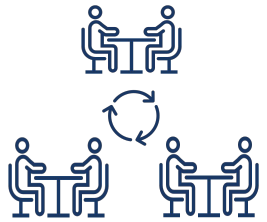
Facilitated by **synergy**, **mutual support** and **prior work** done

New treatment

The Solution: Systematic Serendipity

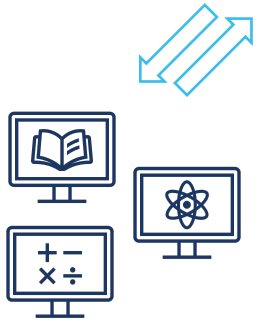
Systematic Serendipity

Increasing the likelihood of identifying treatments through structured **speed-dating** partnering meetings, **database searches**, both **Human** and **Algorithmic**.



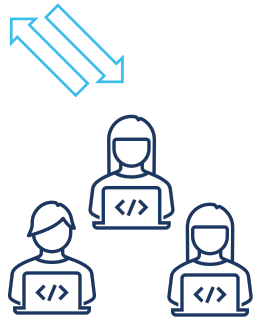
Speed dating

- Patient group team
- Pharma team
- Guided touchpoints
- Clinical/scientific expert



Machine Searches

- Match needs
- Suggest opportunities to explore



Human Searches

- Patient groups & their advisors
- Pharma/biotech
- Researchers
- Entrepeneurs

The Speed Dating Partnering Concept

Patient Group team

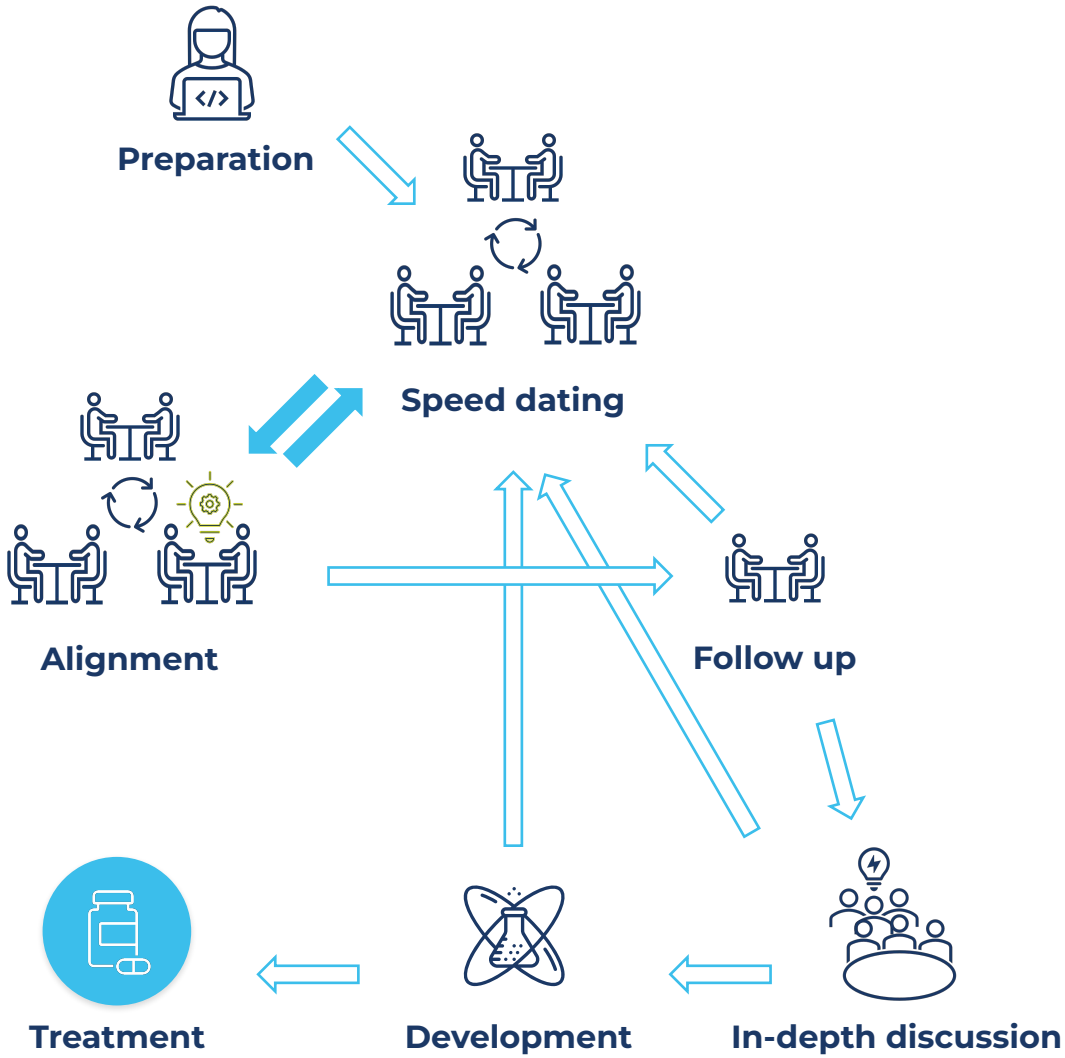
- Familiarity with patient perspective/needs - Advocate/CEO
- Disease mechanism/pathway expertise –
  e.g., scientific/medical trustee/advisor

Company team

- Decision making, strategy –
  e.g., management/patient advocacy
- Familiarity with portfolio/compound library, repurposing candidates and “junkyard” – e.g., CMO, scientist, or clinician

Layout

- Preparation phase
- Patient Group Pitches: * Who You Are * The Condition * *Treatment Options (existing/upcoming) * Unmet Needs *
- Structured Speed Dating, along the **Systematic Serendipity framework**
- Internal debrief
- Guided follow-up touchpoints
- In-depth discussions of selected opportunities



Our Latest Event: Rare Neurology & CNS Cohort

Participants

- Included **8 company teams** from across 3 continents (smallest: 5 employees; largest: 38,000 employees). Each team included a strategy person and technology person.
- Cohort of **9 patient group teams** representing **35 neurological diseases**. Each patient group brought a clinical/research advisor.

Timelines

- Teams built, selected and prepared over 3-4 months (Summer-Fall 2022)
- Companies and patient groups got preparation kits 2-weeks prior to the event (November 2022)
- Partnering event itself: full day at the Royal Society of Medicine in London (November 28th, 2022)
- Currently In-depth follow-up discussions going on

On the day

- Patient Group Pitches
- Company Introductions
- Structured Speed Dating, along the **Systematic Serendipity** framework
- Internal debrief
- Follow-up meetings on the day, on participants request
- Networking

Outcomes:
Rare Neurology & CNS Cohort

Preparation
- **Two-weeks** preparation proves highly effective

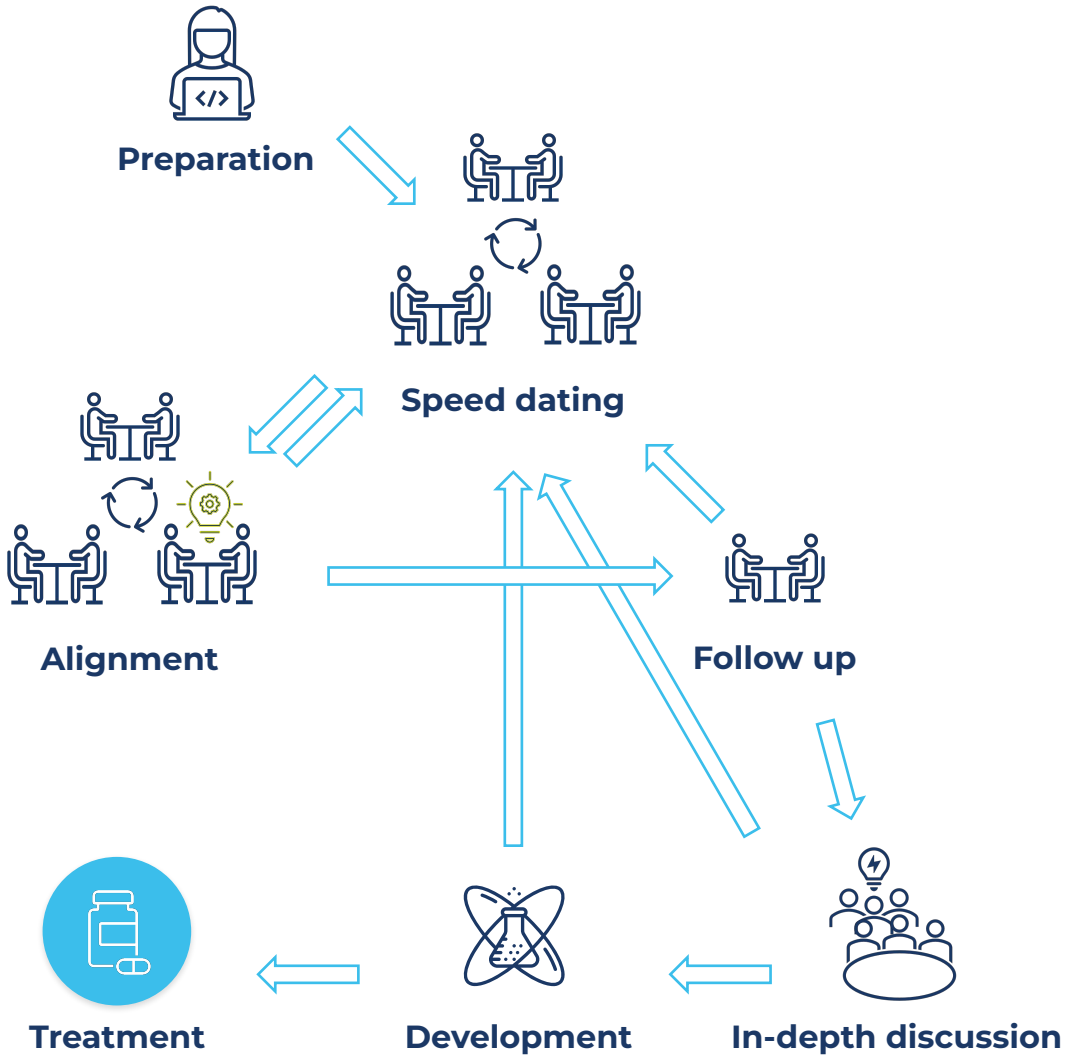
Speed dating
- **72 partnering meetings** on the day

Follow up
- **28 requested follow up meetings** on the day

In-depth discussions
- **14 discussions ongoing** post-event*
- **4 external stakeholders** engaged in discussions*

Development
- **2 CDAs***
- **1 Material Transfer Agreement***
- **1 trial protocol** being adapted*

* As of 6 weeks post event (including New Year's break)

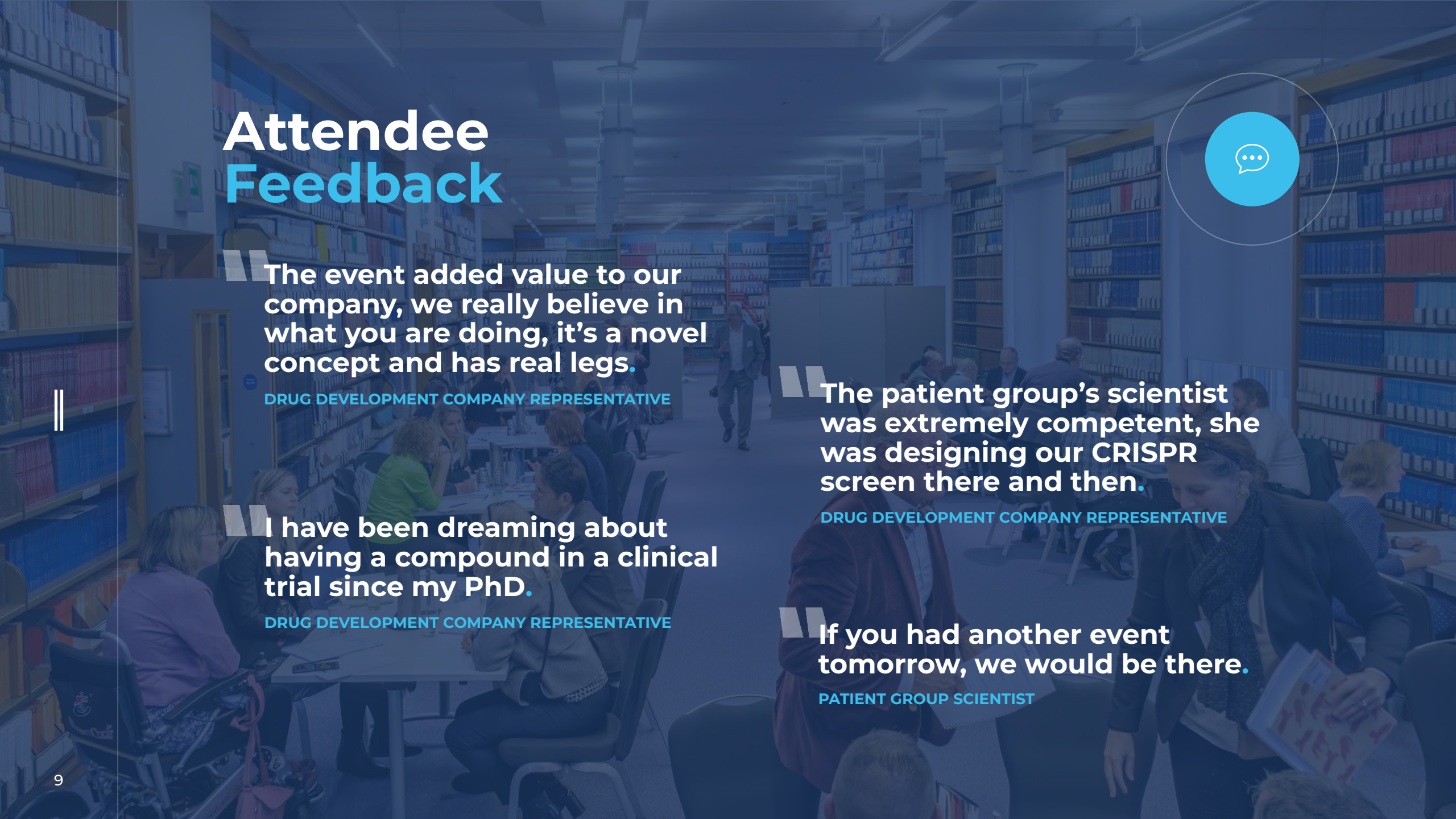


Attendee Feedback

100% of respondents said they would attend another Aspire Biosciences event.

The feedback we received was excellent, with the event's format and organisation universally praised.

Attendee Feedback



> The event added value to our company, we really believe in what you are doing, it's a novel concept and has real legs.

DRUG DEVELOPMENT COMPANY REPRESENTATIVE

> I have been dreaming about having a compound in a clinical trial since my PhD.

DRUG DEVELOPMENT COMPANY REPRESENTATIVE

> The patient group's scientist was extremely competent, she was designing our CRISPR screen there and then.

DRUG DEVELOPMENT COMPANY REPRESENTATIVE

> If you had another event tomorrow, we would be there.

PATIENT GROUP SCIENTIST

Aspire Biosciences
How We Work

Systematic Serendipity

Increase the likelihood of identifying treatments through **speed-dating** partnering meetings, **database searches**, both **Human** and **Algorithmic**

Continuous Discovery

Iterative, **fruitful interplay** between **partnering meetings** and **database searches** to identify opportunities (***de novo*** development, **label expansions**, **repositioning** "junkyard assets", **repurposing**)

Reduce Cost, Increase Benefit

Reduce search costs associated with discovery and engagement overhead, making treatment development viable

Partnership Creation

Nurture working partnerships between patient groups and companies **around identified opportunities**

Viable New Treatments

For unaddressed rare diseases

Our Value

For Drug Development Companies

- Introduction to **new targets** and **previously unknown conditions**
- Finding **repositioning opportunities** for otherwise dead assets, generating new revenue at a relatively low risk.
- **Reduced overhead and costs** associated with patient group discovery, search costs and event management
- Driving **change for good**, increasing **brand value**, demonstrating **social responsibility**

For Patient Groups

- Increased likelihood of **finding a treatment** and companies willing to take on research
- **Raising awareness** to the condition and patient needs
- Improving the organisation's **network and impact** (industry, other patient groups, etc.)

Let’s rethink rare disease treatment, together.

Talk to us about:

- Joining our upcoming partnering event (2nd Rare Neurology cohort, April 2023)
- Getting on board the database we are building
- Additional events in our pipeline: Gene and Cell Therapies, Liver and Metabolic, Rare Hematology – TBA soon

Thank you

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