



THE UPSTAZA™ JOURNEY

Delivering hope through innovation

INVITATION



22 September 2024



1.30pm Registration

2.00pm – 8.30pm Educational Meeting and Dinner



Regent Taipei – 3F Ballroom

No. 3, Ln. 39, Sec. 2 ZhongShan N. Rd.,
Taipei 104, Taiwan



To register, please scan the QR code or go to:

<https://meetings.medkom.com.au/upstaza/register>



A link to the prescribing information and adverse events reporting can be found at the end of this document.

Dear Delegate,

We are pleased to formally invite you to attend 'The Upstaza™ Journey' – Delivering Hope Through Innovation'.

This meeting will provide an overview of Upstaza™, a novel targeted gene therapy for the treatment of AADC deficiency. The topics you will hear more about are:

- Gene therapy discovery and vector development
- Clinical applications of gene therapy
- An overview of Aromatic L-Amino-acid Decarboxylase (AADC) deficiency
- The Upstaza clinical development program and key clinical outcomes
- Best practices for patient management strategies following Upstaza administration
- Real world patient and caregiver experiences with AADCd and Upstaza treatment
- Screening strategies for early identification of patients with AADC deficiency

We look forward to you joining us for this groundbreaking educational and scientific symposium!

Best wishes,

The PTC Therapeutics and Giddi Pharma Team

To confirm your attendance

REGISTER NOW

Time	Topic
13:30-14:00	Registration and Refreshments Outside Ballroom
14:00-14:15	Welcome and Upstaza Overview Dr Matthew Klein
14:15-14:20	Introductions Prof Wuh-Liang Hwu
14:20-14:50	Adeno-associated viral vectors – characterisation and manufacture Prof Shin-ichi Muramatsu
14:50-15:10	Gene therapy with adeno-associated viral vectors Prof Barry Byrne
15:10-15:30	How to diagnose AADC deficiency in the clinic Dr Tung-Ming Chang
15:30-15:50	Upstaza for AADC deficiency Prof Wuh-Liang Hwu
15:50-16:05	Q&A/Panel Discussion Moderator: President Fuu-Jen Tsai
16:05-16:15	Faculty & Group Photos All attendees
16:15-16:30	Afternoon Break
16:30-16:50	Robotic surgery for Upstaza administration Dr Sheng-Che Chou
16:50-17:05	The Upstaza treatment journey: a family perspective Richard Poulin and Judy Wei
17:05-17:45	Gene therapy for AADC deficiency around the globe; France: Prof Agathe Roubertie* Japan: Prof Takanori Yamagata
17:45-18:05	AADC deficiency: newborn screening and post-Upstaza care Prof Yin-Hsiu Chien
18:05-18:25	Q&A/Panel Discussion Moderator: President Kuang-Lin Lin
18:25-18:30	Closing Address PTC Therapeutics
18:30-19:00	Pre-dinner reception and networking
19:00-20:30	Dinner Ballroom

* Via Virtual Platform

If you have any questions, please contact your PTC or Giddi representative.

For any enquiry relating to the processing of your personal data, or if you want to exercise your privacy-related rights or withdraw your consent, you can contact PTC's Privacy Officer at dataprivacy@ptcbio.com.

AADC, aromatic L-amino acid decarboxylase.

This meeting is organised and funded by PTC Therapeutics International Ltd and is intended for healthcare professionals only.

Upstaza™ (eladocagene exuparvovec) is indicated for the treatment of patients aged 18 months and older with a clinical, molecular and genetically confirmed aromatic L-amino acid decarboxylase (AADC) deficiency with a severe phenotype. Upstaza™ is approved for use in the European Member States, Great Britain, Iceland, Liechtenstein, Norway, Northern Ireland and Taiwan.

This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system. Adverse events should also be reported to Giddi Pharma at pv@giddi.com.tw.

Registration conditions differ internationally; always consult local prescribing information and/or the Summary of Product Characteristics before prescribing any product. For the Taiwan Upstaza Package Insert please **click here**. For additional information, please contact Giddi Pharma Co, Ltd. Medical Information on mi@giddi.com.tw

Upstaza™
(eladocagene exuparvovec)

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