Compounded tiratricol for 7 AHDS children at about ₹2.5 lakh a year

Topics: Health · UPSC Prelims · TNPSC · SSC · Current Affairs



News

Through the efforts of Hyderabad paediatrician Dr Sivaranjani Santosh, seven Indian children with Allan–Herndon–Dudley syndrome (AHDS, MCT8 deficiency), a very rare genetic disorder, are to get a low-cost medicine. The branded form of the drug, tiratricol (brand Emcitate), belongs to Sweden's Egetis Therapeutics and costs ₹4.68 crore to ₹9 crore per child a year. Since tiratricol itself is a known thyroid hormone metabolite, the Transvaal Pharmacy in the Netherlands is compounding it with help from the Netherlands-based Pharmaceutical Accountability Foundation (PAF), cutting the cost to about ₹2.5 lakh per child a year. One year's supply for all seven costs about ₹15.08 lakh: Dr Sivaranjani raised ₹6.09 lakh in India and PAF is raising the rest; Access to Medicines India and the Organization for Rare Diseases India (ORDI) also helped. On 7 September 2026 she made a social-media appeal, tagging the Prime Minister and the Union Health Minister. Five of the seven children were identified at CMC Vellore. The medicines are to be handed to the parents on 30 November 2026; she says she will next work for domestic production. The price of the branded drug Emcitate has not been cut; what has been arranged is a low-cost alternative.

Key facts for the exam

Point	Details
Doctor	Dr Sivaranjani Santosh, paediatrician, Hyderabad

Point	Details
Disease	Allan–Herndon–Dudley syndrome (MCT8 deficiency)
Drug	Tiratricol (Emcitate – Egetis, Sweden)
Branded cost	₹4.68–9 crore per child a year
Alternative	Transvaal Pharmacy, Netherlands – ~₹2.5 lakh a year
Total (7 children)	About ₹15.08 lakh (₹6.09 lakh raised in India)
Partners	PAF (Netherlands), Access to Medicines India, ORDI
Handover	30 November 2026 (scheduled)

Background

- The disease: AHDS is an X-linked genetic disorder caused by mutations in the MCT8 (SLC16A2) gene. MCT8 carries thyroid hormone into brain cells; when it fails, the brain is starved of the hormone, causing developmental delay, intellectual disability and low muscle tone, while blood T3 rises.
- X-linked inheritance: males have a single X chromosome, so a faulty gene on it shows up as disease; in females the second X usually compensates, so they are mostly carriers. Haemophilia is another such disorder.
- How the drug works: tiratricol (TRIAC) enters cells without MCT8 and binds thyroid receptors, bringing down blood T3. It must be taken for life; with a six-month shelf life it will be imported in two batches.
- Orphan drugs and price: medicines for rare diseases that affect very few people are called 'orphan drugs'; small markets make them very costly. For a long-known substance, routes such as pharmacy compounding can offer a cheaper alternative. India's New Drugs and Clinical Trials Rules, 2019 (rule 2(x)) define an orphan drug as one for a condition affecting not more than 5 lakh persons in India. Section 3(d) of the Patents Act, 1970 denies patents to new forms of a known substance unless efficacy is enhanced; the Supreme Court applied it in the 2013 Novartis (Glivec) case.

Tamil Nadu link: Five of the seven children were identified at, and are treated by, Christian Medical College (CMC), Vellore; CMC doctors were among those Dr Sivaranjani consulted.

Frequently asked questions

What is this news about?

Through the efforts of Hyderabad paediatrician Dr Sivaranjani Santosh, seven Indian children with Allan–Herndon–Dudley syndrome (AHDS, MCT8 deficiency), a very rare genetic disorder, are to get a low-cost medicine.

What are the key facts to remember for the exam?

Doctor: Dr Sivaranjani Santosh, paediatrician, Hyderabad; Disease: Allan–Herndon–Dudley syndrome (MCT8 deficiency); Drug: Tiratricol (Emcitate – Egetis, Sweden); Branded cost: ₹4.68–9 crore per child a year.

What is the background to this news?

The disease: AHDS is an X-linked genetic disorder caused by mutations in the MCT8 (SLC16A2) gene. MCT8 carries thyroid hormone into brain cells; when it fails, the brain is starved of the hormone, causing developmental delay, intellectual disability and low muscle tone, while blood T3 rises.

Practice questions (with answers)

1. In Allan–Herndon–Dudley syndrome, a faulty MCT8 protein stops which hormone from entering brain cells?

A) Insulin

B) Thyroid hormone

C) Growth hormone

D) Cortisol

Answer: B – Explanation: Thyroid hormone, vital for brain development. Tiratricol acts on thyroid receptors without needing MCT8.

2. Which section of the Patents Act, 1970, applied in the 2013 Novartis case, denies patents to new forms of a known substance without enhanced efficacy?

A) Section 84

B) Section 53

C) Section 3(d)

D) Section 92

Answer: C – Explanation: Section 3(d) – Novartis was denied a patent for its cancer drug Glivec. Section 84: compulsory licence; Section 53: term of patent.

3. Christian Medical College (CMC), where five of these seven children were identified, is in which Tamil Nadu city?

A) Vellore

B) Madurai

C) Coimbatore

D) Tiruchirappalli

Answer: A – Explanation: Vellore – Dr Sivaranjani also consulted the CMC doctors treating these children.

4. AHDS is X-linked. Why do such disorders mostly affect boys?

A) Faulty Y chromosome

B) Males have one X

C) Less breastfeeding

D) Excess hormones

Answer: B – Explanation: Males have one X, so nothing offsets the fault; in females the second X compensates, making them mostly carriers. Haemophilia is similar.

5. Under the New Drugs and Clinical Trials Rules, 2019, a drug for a condition affecting not more than 5 lakh persons in India is called a/an:

A) Generic drug

B) OTC drug

C) Biosimilar

D) Orphan drug

Answer: D – Explanation: Orphan drug – rule 2(x); small markets make them very costly. Generics copy off-patent drugs; OTC drugs need no prescription.