Mepsevii access in India

How Indian families with mucopolysaccharidosis type VII (MPS VII, Sly syndrome) pursue Mepsevii (vestronidase alfa), an enzyme replacement therapy for one of the rarest lysosomal storage disorders.

Last reviewed 2026-05-12 by Reserve Meds clinical & regulatory team. This page combines the India country regulatory module with the Mepsevii drug module to describe the path families actually walk.

Quick orientation

Mepsevii (vestronidase alfa) is a recombinant human beta-glucuronidase enzyme replacement therapy approved by the US Food and Drug Administration in November 2017 for mucopolysaccharidosis type VII (MPS VII, also known as Sly syndrome), an ultra-rare lysosomal storage disorder. The product is marketed approximately by Ultragenyx Pharmaceutical. Mepsevii is delivered by intravenous infusion every two weeks at a recommended dose of 4 mg per kg. MPS VII affects an estimated 1 in 250,000 to 1 in 1,000,000 live births globally, putting the worldwide patient population in the low hundreds and the Indian patient population in the very low double digits or single digits. Reserved for you.

Why this drug is hard to source in India

The access wall for Mepsevii in India is the natural consequence of how ultra-rare orphan therapies launch. The originator manufacturer has not pursued an Indian marketing authorisation through CDSCO because the Indian patient population is too small to support a commercial launch. The drug is approved by the FDA, EMA, and selected reference authorities. For any Indian family with a confirmed MPS VII diagnosis, access runs entirely through the import pathway and the National Policy for Rare Diseases framework. The Indian rare-disease ecosystem at AIIMS Delhi, the Centre for Genetic Disorders at AIIMS, and metabolic genetics units at major academic institutions has documented diagnostic capability for the MPS family of disorders including MPS VII through urinary glycosaminoglycan analysis and beta-glucuronidase enzymatic activity testing, with genetic confirmation through GUSB gene sequencing.

The CDSCO personal-import pathway and Compassionate Use

For unregistered ultra-rare orphan drugs not approved for marketing in India at all, the institutional Compassionate Use route to the DCGI is generally used in parallel with or instead of the Form 12A Rule 36 personal-import pathway. The Compassionate Use route allows a government hospital, a registered medical practitioner, a pharmaceutical company, or the patient to seek access to an unregistered drug for a life-threatening condition, a serious permanent disability, or an unmet medical need, where the drug is approved by a recognised reference authority such as the FDA, EMA, MHRA, Health Canada, or PMDA. AIIMS and Tata Memorial Centre have established workflow for this pathway. For Mepsevii specifically, the Compassionate Use submission is anchored on the FDA approval, the unmet need (no other approved enzyme replacement therapy for MPS VII), and the institutional capability to administer biweekly intravenous infusions and manage potential infusion reactions.

Real costs in INR and USD

The US wholesale acquisition cost for Mepsevii is approximately USD 375,000 to 450,000 per year for a typical pediatric or adolescent patient on biweekly dosing, with significant variation by body weight because dosing is weight-based at 4 mg per kg. In INR at the prevailing 94 to 95 range, that translates to approximately INR 3.5 crore to 4.2 crore per year. An Indian-made generic or biosimilar of vestronidase alfa does not exist as of this review date. The biologic complexity of a recombinant human enzyme produced for an ultra-rare indication does not support a domestic generic pathway. The National Policy for Rare Diseases 2021 designates lysosomal storage disorders including the MPS family as eligible categories, with the Rashtriya Arogya Nidhi umbrella scheme providing one-time financial assistance up to INR 50 lakh per patient. The Mepsevii annual cost meaningfully exceeds the NPRD ceiling, and families typically combine NPRD coverage where granted with cash payment, foundation grants, and where applicable foreign-funded support routed through compliant channels.

Timing, what to expect

From diagnosis confirmation to first infusion, a Mepsevii case typically runs four to eight weeks because the Compassionate Use submission to DCGI is more involved than a routine Rule 36 application. Documentation assembly including the enzymatic activity report, the genetic confirmation, the institutional infusion capability statement, and the manufacturer disposition takes one to two weeks. The DCGI review for Compassionate Use is case-by-case and not on the one to two day Form 12B priority timeline. US-side sourcing and

shipment runs one to two weeks. For ongoing biweekly infusions, Reserve Meds aligns supply cycles to a monthly reorder rhythm.

What your physician needs

The clinical justification letter documents the MPS VII diagnosis with enzymatic activity confirmation (deficient beta-glucuronidase activity in peripheral blood leukocytes or fibroblasts) and genetic confirmation (pathogenic variants in the GUSB gene), the patient's clinical phenotype (skeletal, neurological, ophthalmologic, cardiac, and respiratory manifestations), the weight-based dose calculation at 4 mg per kg biweekly, the institutional infusion capability including pre-medication protocol for infusion reactions, and the planned monitoring schedule including urinary glycosaminoglycan response. The treating metabolic genetics specialist's NMC registration number appears on the prescription.

Customs clearance and IOR

Customs at the port of entry reviews the Compassionate Use authorisation or Form 12B permit, the commercial documentation, the airway bill, the cold-chain monitoring record, and the importer's drug licence. The Importer of Record is the licensed dispensing facility, typically the tertiary academic hospital where the infusions will be administered. Mepsevii is stored refrigerated at 2 to 8 degrees Celsius; cold-chain integrity is documented through the transit monitor.

Pharmacovigilance

India operates the Pharmacovigilance Programme of India (PvPI) coordinated by the Indian Pharmacopoeia Commission. For Mepsevii, the principal post-marketing safety question is infusion-related reactions and anaphylaxis, with the FDA label carrying a boxed warning. The infusion facility maintains a pre-medication protocol and immediate management capability. The PvPI reference is included in the Reserve Meds physician documentation kit and adverse event reporting is the prescribing physician's responsibility.

Where Reserve Meds fits

Reserve Meds is a US-based concierge coordinator. For ultra-rare cases like MPS VII, our role is US-side sourcing of an orphan biologic, documentation orchestration for the Compassionate Use submission to DCGI, cold-chain logistics, and a single named coordinator across the multi-week initial cycle and the ongoing biweekly supply cadence. We coordinate with the rare-disease Centre of Excellence handling the case.

Next step

If a treating metabolic genetics specialist in India is weighing Mepsevii for a patient with confirmed MPS VII, the waitlist is the first step. We respond within 24 to 48 hours with an eligibility confirmation and a documentation kit for the physician.

Reserved for you.

Related

- Mepsevii clinical resource
- India country page
- CDSCO personal-import pathway

Sources

1. FDA approval, Mepsevii (vestronidase alfa), Ultragenyx Pharmaceutical, BLA approval November 2017 for MPS VII (Sly syndrome).
2. CDSCO, Compassionate Use of Unapproved Drugs regulatory framework for life-threatening conditions and unmet medical need.
3. National Policy for Rare Diseases 2021, Rashtriya Arogya Nidhi umbrella scheme financial assistance framework.

Common questions Indian families ask

Will Star Health, HDFC ERGO, ICICI Lombard, or Niva Bupa cover this? Each Indian private insurer assesses named-patient imports case by case. None of the major private insurers reimburse a Rule 36 personal import as a standard line item. Some have reimbursed full or partial drug cost where the underlying medicine is on the formulary and the named-patient route operated as a stocking workaround. Reserve Meds supplies the documentation that lets your insurer evaluate. The claim itself is filed by the patient or the hospital. Cash-pay remains the default posture for Reserve Meds patient cases.

Will my CGHS or ESIC entitlement cover this? CGHS (Central Government Health Scheme) provides for life-saving and anti-cancer medicines not in the standard formulary to be considered case by case by an Expert Committee under the Special DG (DGHS) where the prescribing specialist documents the requirement. Drugs not approved by the DCGI for use in India face a stricter Expert Committee review. ESIC's formulary is narrower. Neither scheme is structured for routine personal-import reimbursement; check eligibility with your CGHS Wellness Centre or the ESIC dispensary before assuming coverage.

Will my AIIMS, Tata Memorial, Apollo, Fortis, Medanta, Kokilaben, MGM, CMC Vellore, or Manipal physician's letter be sufficient? Yes. A Registered Medical Practitioner with a valid National Medical Commission registration number can support a Form 12A application. Physicians at AIIMS, Tata Memorial Centre, government medical colleges, and state-run tertiary hospitals routinely do so. Private-sector specialists at Apollo, Fortis, Medanta, Kokilaben Dhirubhai Ambani Hospital, MGM Healthcare, CMC Vellore, and Manipal Hospitals also have signing authority subject to their institutional drug licence.

What if my treating institution does not have an import pharmacy desk? The practical route is to work with one of the named tertiary centres that handles compassionate and named-patient imports as established workflow, or with a CDSCO-licensed specialty importer in Mumbai, Delhi, or Bangalore that handles the documentation and chain-of-custody on behalf of smaller hospitals or independent specialists. Reserve Meds aligns with the importer named on the Form 12B permit.

Can I receive the drug at home, or do I need a hospital? The dispensing facility must hold a valid drug licence under the Drugs and Cosmetics Rules. For oral medicines, a hospital outpatient pharmacy or a licensed import pharmacy is the dispensing point. For infusion products, the medicine ships to the infusion centre where the patient will receive it. Direct-to-home delivery outside a licensed dispensing facility is not the model.

What about pediatric patients? The Rule 36 framework applies the same way for pediatric patients. The clinical justification letter typically includes weight-adjusted dosing and pediatric-specific monitoring. AIIMS, Tata Memorial, Apollo, Kokilaben, and CMC Vellore handle pediatric named-patient imports routinely. Where the indication is approved in adults only, the off-label use is the physician's clinical judgement and is documented as such in the Form 12A letter.

Does FCRA affect a patient case? The Foreign Contribution (Regulation) Act 2010 (FCRA), as proposed to be amended by the Foreign Contribution (Regulation) Amendment Bill 2026, regulates foreign donations to Indian organisations and individuals. For a patient family paying for the medicine themselves, FCRA is generally not engaged. Where a foreign foundation or diaspora group is funding a treatment, FCRA registration of the recipient organisation and the donation route can become relevant; the structure should be reviewed with counsel before funds move. Reserve Meds does not provide FCRA legal advice; we flag the question so it reaches the right adviser early.

What is the role of the Indian Pharmacopoeia Commission? The Indian Pharmacopoeia Commission coordinates the Pharmacovigilance Programme of India (PvPI) and publishes the Indian Pharmacopoeia, the legal compendium of pharmaceutical standards in India. For imported originator products under Rule 36, the PvPI reporting framework applies to adverse event surveillance, and the prescribing physician is the reporting party. The Indian Pharmacopoeia is the reference standard against which Indian-manufactured products are

tested; imported originator products carry their reference-country pharmacopoeial standards (typically USP for FDA-approved products).

How does this compare with access in the UAE or Saudi Arabia? India's Rule 36 framework with the published Form 12B priority timeline (one to two business days for routine documentation) is often faster than the SFDA Personal Importation Program in Saudi Arabia (typically 10 to 21 business days routine) and broadly comparable to the UAE Emirates Drug Establishment pathway. India's offsetting friction is the customs and logistics layer rather than the regulator's stamp. India's tertiary specialty hospital depth substantially exceeds any single peer country in the South Asia and GCC region, which usually offsets the longer end-to-end cycle for complex cases.

What documentation does my family need to assemble before contacting Reserve Meds? The minimum useful package is the treating physician's name and registration number, the patient's diagnosis and current treatment summary, recent relevant investigations (imaging, biopsy, molecular pathology, blood work as applicable), and a contact pathway to the dispensing facility you intend to use. With that package, Reserve Meds can complete eligibility within 24 to 48 hours and route the documentation kit to your physician.

How Indian families coordinate across cities and countries

For Indian families, the coordination problem is often distributed across multiple cities and sometimes multiple countries. A grandmother in Hyderabad, an oncologist at Tata Memorial in Mumbai, an adult child in Bangalore managing logistics, and a son in Dubai or London paying the invoice is a common configuration. The Reserve Meds single named coordinator model is built for exactly this pattern. One coordinator carries the case file, one chain of correspondence captures the decisions and documents, and one set of contact records reaches every family member who needs visibility into the case, regardless of how many cities the family touches or how many time zones the case spans. The Reserve Meds patient portal at portal.reservemeds.com holds the document set and the case timeline; the coordinator handles the email, phone, and WhatsApp follow-through that the case needs at each step.

For smaller cities where the local hospital does not maintain an import pharmacy desk, the practical route is to work with a CDSCO-licensed specialty importer in Mumbai, Delhi, Bangalore, Chennai, or Hyderabad. The importer carries the CDSCO relationship, the customs broker relationship, and the chain-of-custody documentation. Reserve Meds aligns with the importer on US-side sourcing and with the treating physician on clinical documentation. The patient sees one face throughout, which is the named coordinator.

The patient experience, step by step

From the family side, the sequence looks like this. Your physician decides this drug is the right next step. That is a clinical decision and stays with them. Your physician or the hospital pharmacy team reaches out to Reserve Meds, or the patient submits a request through the Reserve Meds portal and Reserve Meds connects with the physician. Reserve Meds confirms eligibility within 24 to 48 hours and sends a documentation kit to your physician, including the Form 12A reference, the clinical justification letter template, and the chain-of-custody plan. Your physician completes the documentation, attaches the prescription with their NMC registration number, and the application goes to CDSCO through the appropriate port office or the DCGI New Delhi office, or via the hospital's licensed importer. The Form 12B permit issues on the documented priority timeline. While the permit issues, Reserve Meds aligns US-side sourcing and the shipment plan with the dispensing pharmacy. The shipment moves cold-chain or ambient as appropriate. Customs at the destination port reviews the permit and clears the consignment. The dispensing pharmacy receives, logs, and stores the medicine according to its drug licence requirements. Your physician initiates therapy. Adverse event reporting through PvPI continues for the duration of therapy.

Review and oversight. Content on this page is reviewed by the Reserve Meds clinical and regulatory team. A US-licensed pharmacist reviews every prescription before dispensing. Regulatory posture is informational, not legal advice; case-specific questions route to retained outside counsel. Review methodology ›
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