International Patient Advocacy Group Meeting

2023 ANNUAL SMA CONFERENCE

Challenges in Standard of Care and Access Wins

Alpana Sharma / Moumita Ghosh

cure SMA

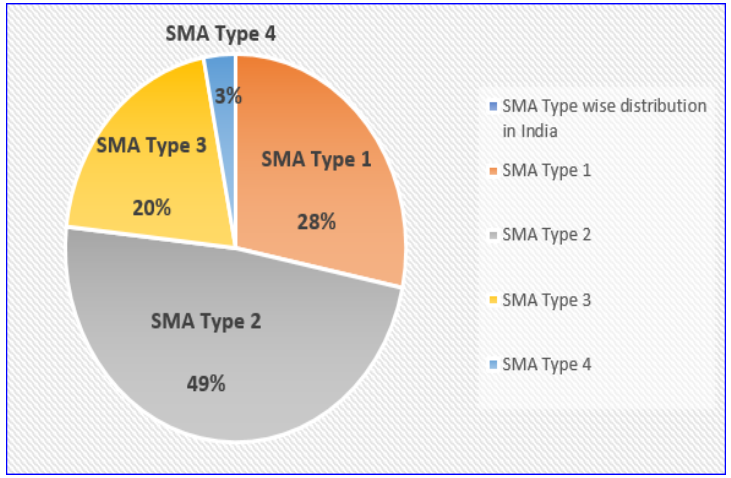
Cure SMA Foundation of India

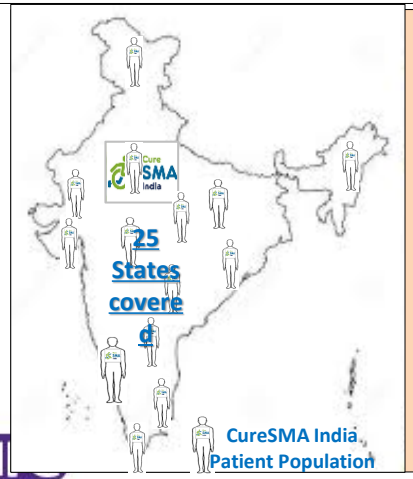
Who we are

CureSMA India is a parent led Non Profit Registered Organization since 2014.

We represent around 1100+ registered patients, mostly children battling the life-threatening neuromuscular genetic disorder Spinal Muscular Atrophy.

We represent ALL SMA Types





Prevalence & Incidence of SMA in India.

Current live births in India 67385

(source UNICEF India, https://www.unicef.org/india/key-data),

1 SMA child is born in every 2 to 2.5 hours in India

11 SMA children are born every day in India

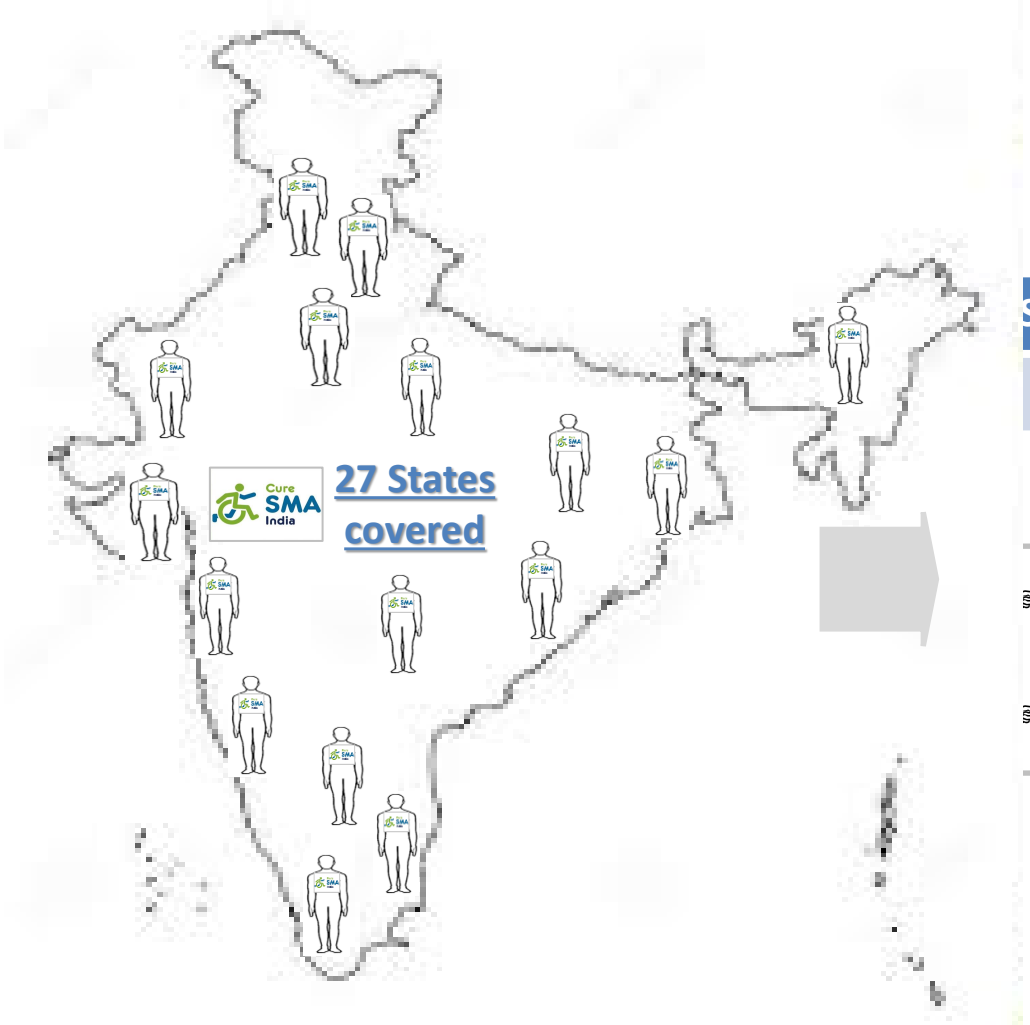
4015 SMA children are born every year in India

VISION & MISSION

We dream of an SMA free world and a barrier free universal healthcare for all.

We believe in working to co-create solutions

CureSMA India - Our Geographic Coverage, Patient Population



Approx. 1100+ registered patients (and increasing.....)

SMA Type 1	SMA Type 2	SMA Type 3	SMA Type 4
10%	63%	25%	2%

(Note: Indicative break-down only)

- Patient registry and on-boarding increasing (diagnosis and discovery +awareness)
- Formalised on-boarding process of CureSMA India

- Critical patients in network
- 48 + lives lost (in the last 6 months)

CureSMA City Co-ordinators

Cure SMA India's Journey so far - POLICY MAKING AND ENGAGEMENT WITH GOVERNMENT OF INDIA

- POLICY MAKING AND ENGAGEMENT WITH- DCGI-MoHFW,
- New Rules for Clinical Trials
- Draft Rules for Compassionate Use Program

[भाग II—खण्ड 3(i)] भारत का राजपत्र : असाधारण 147

MINISTRY OF HEALTH AND FAMILY WELFARE

(Department of Health and Family Welfare)

NOTIFICATION

New Delhi, the 19th March, 2019

G.S.R.227(E) .— WHEREAS the draft of the New Drugs and Clinical Trials Rules, 2018 was published, in exercise of the powers conferred by sub-section (1) of section 12 and sub-section (1) of section 33 of the Drugs and Cosmetics Act, 1940 (23 of 1940), in the Gazette of India, Extraordinary, Part II, section 3, sub-section (i) *vide* notification number G.S.R. 104(E), dated the 1st February, 2018, by the Central Government, after consultation with the Drugs Technical Advisory Board, inviting objections and suggestions from all persons likely to be affected thereby, before the expiry of a period of forty-five days from the date on which copies of the Official Gazette containing the said notification were made available to the public;

AND WHEREAS, copies of the Official Gazette containing the said notification were made available to the public on the 7th February, 2018;

AND WHEREAS, all objections and suggestions received in response to the said draft notification have been duly considered by the Central Government;

AND WHEREAS, the Hon'ble Supreme Court of India in Writ Petition(s) (Civil) No (s). 33/2012 Swathaya Adhikar Manch, Indore and another Versus Union of India and others with W.P.(C) No. 79/2012 (PIL-W), *inter alia,* observed that new clinical trial rules shall be finalised urgently;

NOW, THEREFORE, in exercise of the powers conferred by section 12 and section 33 of the Drugs and Cosmetics Act, 1940 (23 of 1940), the Central Government, after consultation with the Drugs Technical Advisory Board, hereby makes the following rules, namely:—

12 THE GAZETTE OF INDIA : EXTRAORDINARY [PART II—SEC. 3(i)]

MINISTRY OF HEALTH AND FAMILY WELFARE

(Department of Health and Family Welfare)

NOTIFICATION

New Delhi, the 5th June, 2020

G.S.R. 354(E).—The following draft of certain rules to amend the New Drugs and Clinical Trials Rules, 2019 which the Central Government proposes to make, in exercise of the powers conferred by sub-section (1) of section 12 and sub-section (1) of section 33 of the Drugs and Cosmetics Act, 1940 (23 of 1940) and in consultation with the Drugs Technical Advisory Board is hereby published for information of all persons likely to be affected thereby and notice is hereby given that the said draft rules shall be taken into consideration on or after the expiry of a period of fifteen days from the date on which the copies of the Gazette of India containing these draft rules are made available to public;

Objections and suggestions which may be received from any person within the period specified above will be considered by the Central Government;

Objections and suggestions, if any, may be addressed to the Under Secretary (Drugs), Ministry of Health and Family Welfare, Government of India, Room No. 414 A, D Wing, Nirman Bhavan, New Delhi - 110011 or emailed at drugsdiv-mohfw@gov.in.

DRAFT RULES

1. (1) These rules may be called the New Drugs and Clinical Trials (.....Amendment) Rules, 2020.
   (2) They shall come into force on the date of their final publication in the Official Gazette.
2. In the New Drugs and Clinical Trials Rules, 2019,

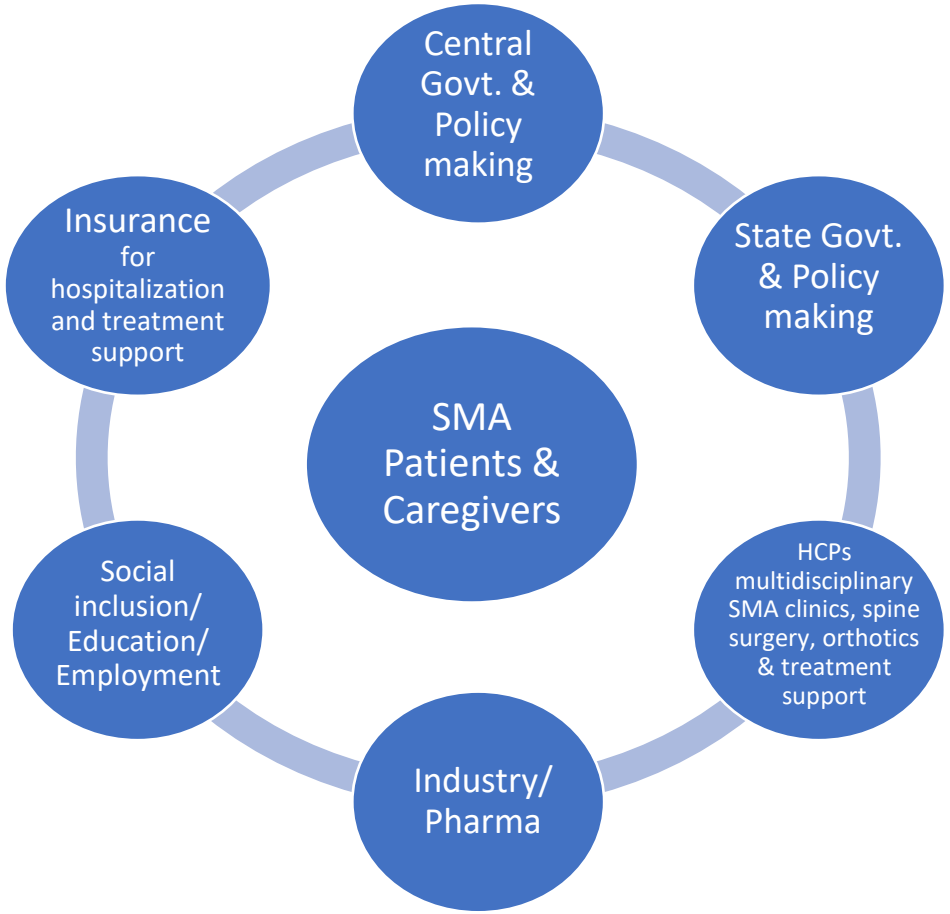
Cure SMA India- Seeking multi stakeholder support in creating pathway for Sustainable healthcare ecosystem for SMA

Short term goal

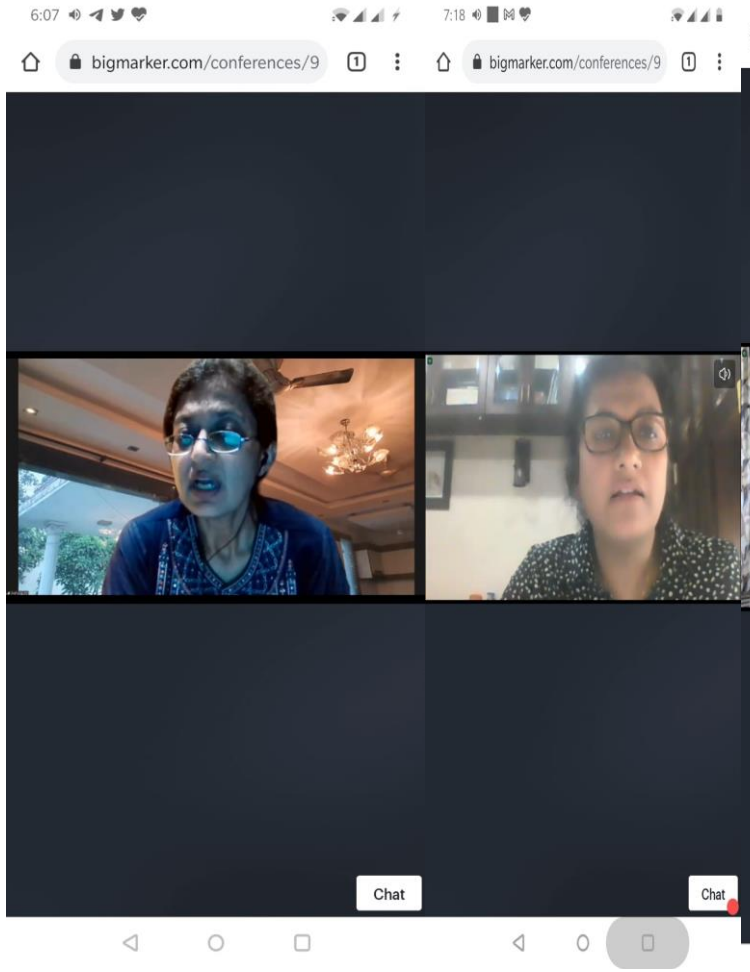
- Management of disease SMA with standard protocols and best practices
- Access to available treatment immediately at affordable rates

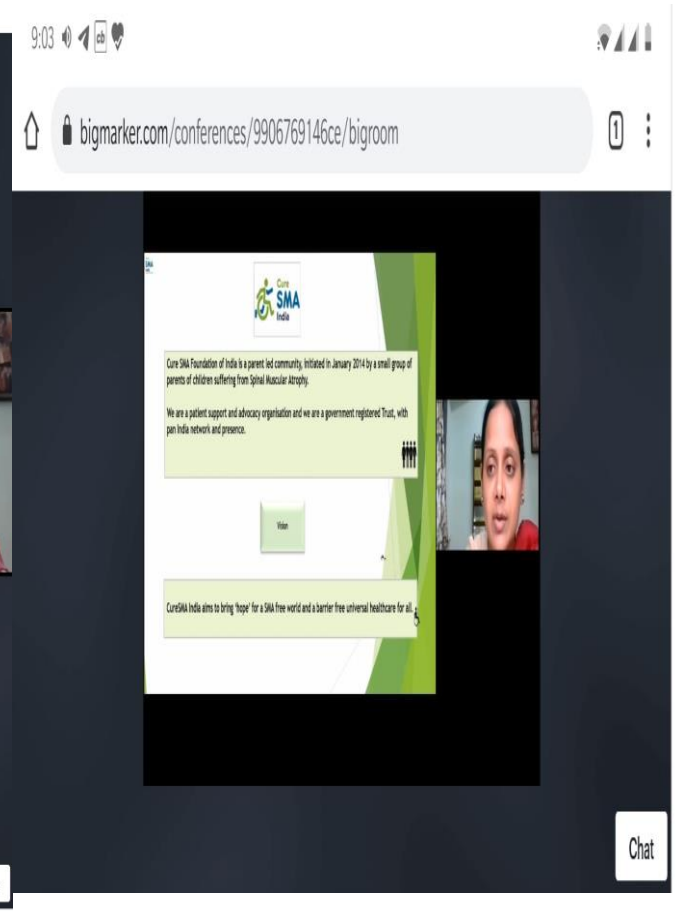
Long term goal

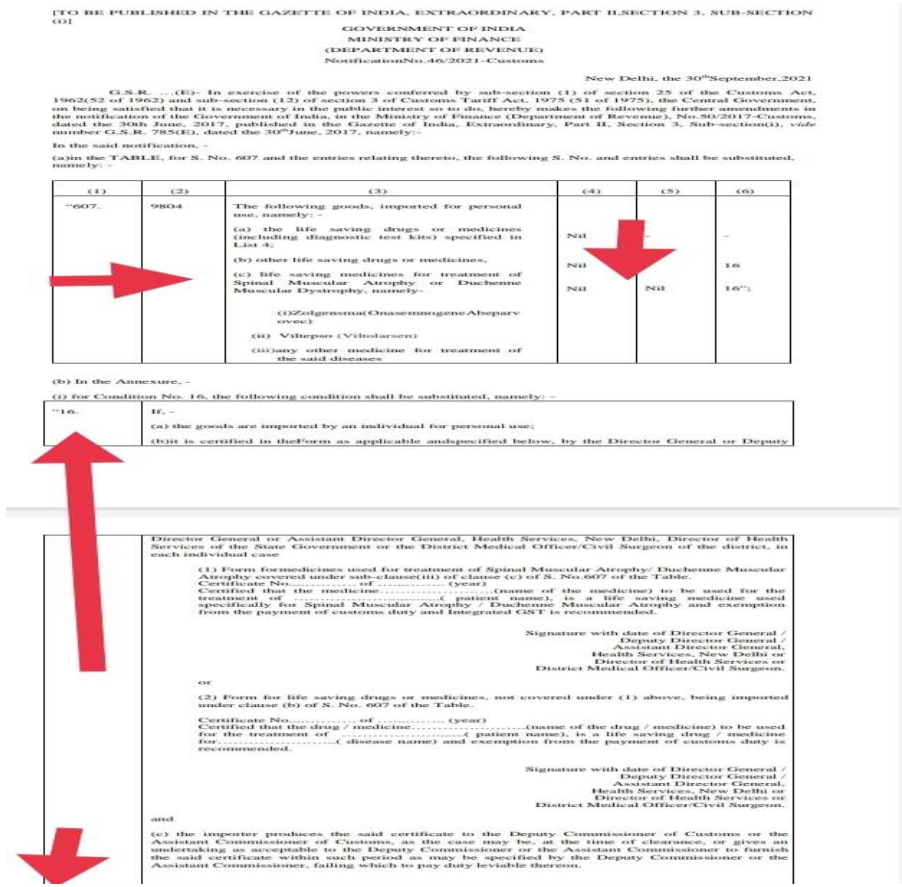
- Life long continued access to affordable treatment
  - CORPORATE FUNDS & GOVT FUNDS
- Care & Management with standard protocols at nearest convenient center.
- Social inclusion, accessibility, education and employment, empowerment & livelihood.
- Genetic screening, new born screening
- Pathway towards early access to life saving medicines to save lives and preserve functionality as much as possible & create a sustainable ecosystem for SMA patients.



RISDIPLAM LAUNCH







[TO BE PUBLISHED IN THE GAZETTE OF INDIA, EXTRAORDINARY, PART II,SECTION 3, SUB-SECTION (i)]

GOVERNMENT OF INDIA
MINISTRY OF FINANCE
(DEPARTMENT OF REVENUE)
NotificationNo.46/2021-Customs

New Delhi, the 30th September,2021

G.S.R. ...(E)- In exercise of the powers conferred by sub-section (1) of section 25 of the Customs Act, 1962(52 of 1962) and sub-section (12) of section 3 of Customs Tariff Act, 1975 (51 of 1975), the Central Government, on being satisfied that it is necessary in the public interest so to do, hereby makes the following further amendments in the notification of the Government of India, in the Ministry of Finance (Department of Revenue), No.50/2017-Customs, dated the 30th June, 2017, published in the Gazette of India, Extraordinary, Part II, Section 3, Sub-section(i), *vide* number G.S.R. 785(E), dated the 30th June, 2017, namely:-

In the said notification, -

(a)in the TABLE, for S. No. 607 and the entries relating thereto, the following S. No. and entries shall be substituted, namely: -

(1)	(2)	(3)	(4)	(5)	(6)
"607.	9804	The following goods, imported for personal use, namely: -			
		(a) the life saving drugs or medicines (including diagnostic test kits) specified in List 4;	Nil	-	-
		(b) other life saving drugs or medicines,	Nil	-	16
		(c) life saving medicines for treatment of Spinal Muscular Atrophy or Duchenne Muscular Dystrophy, namely-	Nil	Nil	16";
		(i)Zolgensma(Onasemnogene Abeparvovec)			
		(ii) Viltepso (Viltolarsen)			
		(iii)any other medicine for treatment of the said diseases			

(b) In the Annexure, -

(i) for Condition No. 16, the following condition shall be substituted, namely: -

"16.	If, -
	(a) the goods are imported by an individual for personal use;
	(b)it is certified in theForm as applicable andspecified below, by the Director General or Deputy Director General or Assistant Director General, Health Services, New Delhi, Director of Health Services of the State Government or the District Medical Officer/Civil Surgeon of the district, in each individual case

(1) Form formedicines used for treatment of Spinal Muscular Atrophy/ Duchenne Muscular Atrophy covered under sub-clause(iii) of clause (c) of S. No.607 of the Table.

Certificate No.....of.....(year)

Certified that the medicine.....(name of the medicine) to be used for the treatment of.....(patient name), is a life saving medicine used specifically for Spinal Muscular Atrophy / Duchenne Muscular Atrophy and exemption from the payment of customs duty and Integrated GST is recommended.

Signature with date of Director General / Deputy Director General / Assistant Director General, Health Services, New Delhi or Director of Health Services or District Medical Officer/Civil Surgeon.

or

(2) Form for life saving drugs or medicines, not covered under (1) above, being imported under clause (b) of S. No. 607 of the Table.

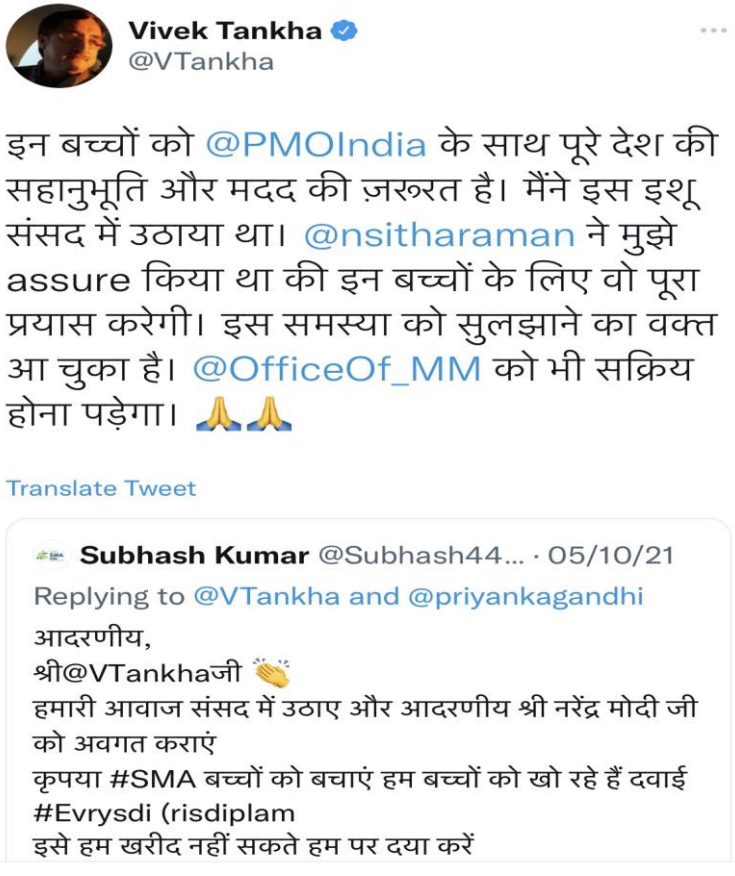
Certificate No.....of.....(year)

Certified that the drug / medicine.....(name of the drug / medicine) to be used for the treatment of .....(patient name), is a life saving drug / medicine for.....(disease name) and exemption from the payment of customs duty is recommended.

Signature with date of Director General / Deputy Director General / Assistant Director General, Health Services, New Delhi or Director of Health Services or District Medical Officer/Civil Surgeon.

and

(c) the importer produces the said certificate to the Deputy Commissioner of Customs or the Assistant Commissioner of Customs, as the case may be, at the time of clearance, or gives an undertaking as acceptable to the Deputy Commissioner or the Assistant Commissioner to furnish the said certificate within such period as may be specified by the Deputy Commissioner or the Assistant Commissioner, failing which to pay duty leviable thereon.



Vivek Tankha
@VTankha

इन बच्चों को @PMOIndia के साथ पूरे देश की सहानुभूति और मदद की ज़रूरत है। मैंने इस इशू संसद में उठाया था। @nsitharaman ने मुझे assure किया था की इन बच्चों के लिए वो पूरा प्रयास करेगी। इस समस्या को सुलझाने का वक्त आ चुका है। @OfficeOf_MM को भी सक्रिय होना पड़ेगा। 🙏🙏

Translate Tweet

Subhash Kumar @Subhash44... · 05/10/21

Replying to @VTankha and @priyankagandhi

आदरणीय,
श्री@VTankhaजी 👏
हमारी आवाज संसद में उठाए और आदरणीय श्री नरेंद्र मोदी जी को अवगत कराएं
कृपया #SMA बच्चों को बचाएं हम बच्चों को खो रहे हैं दवाई
#Evrysdi (risdiplam
इसे हम खरीद नहीं सकते हम पर दया करें

‘Institutionalise import exemptions for SMA kids’

Baby Teera, who suffers from a rare genetic disease, has got an exemption of customs duty and GST waiver worth over ₹5 crore on the import of Zolgensma — a gene therapy medication for SMA

- We have been constantly meeting and advocating with both State and Central Government regarding treatment access and creating robust infrastructure for SMA Patients and families. This has resulted in Custom and Import Duty Exemptions for one of the SMA Drug

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Access Win – Kerala NHM & RACE

Kerala govt. is proving Risdiplam for young SMA patients. The initial procurement is expected to be continued & the govt. may extend the programme to more patients. This is Outcome of the hard work by all of us (CureSMA India especially Dr Razeena, Roche and State NHM)

R – Railways

A- Army & Navy

C – Central govt employees

E - ESI

Strategic Global Partnership

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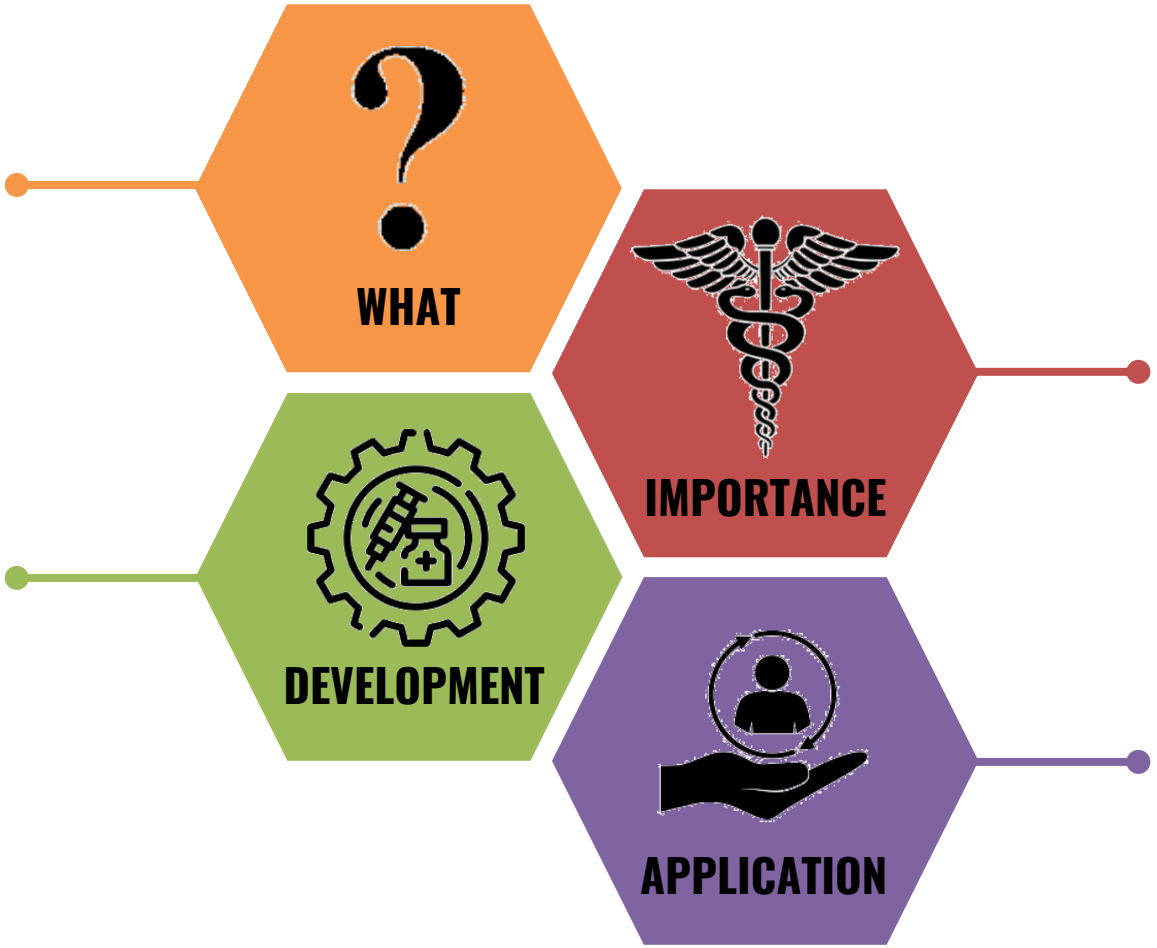
Standard of Care (SoC)

Treatment that is accepted by medical experts as a proper treatment for a certain type of disease and that is widely used by healthcare professionals

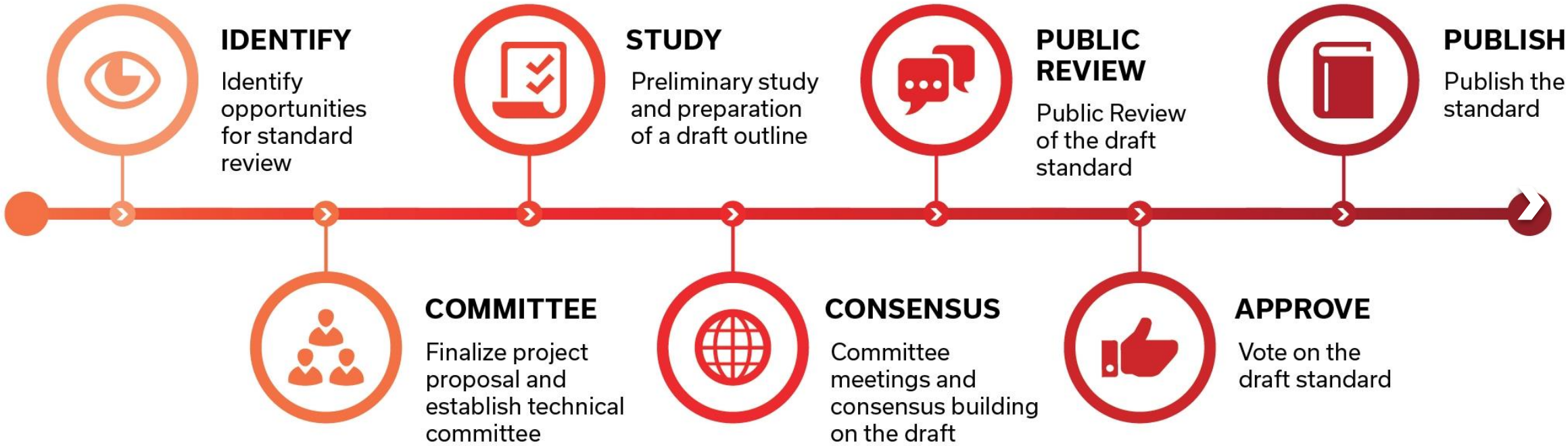
Standards are created or reviewed by experts in the relevant field. They include researchers, care providers, patients and families, who form a technical committee.



The Standard of Care is the benchmark that determines whether professional obligations to patients have been met.

In a rare disease like SMA, following a SoC is important to provide the best palliative and medical care available.

Standard of Care Review & Development Process (Health Standard Organisations Model)



Source: https://healthstandards.org/standards/development-process/

Standard of Care (SoC): Challenges, Solution & Benefits

CHALLENGES

- Low awareness about the condition
- Poor clinical skills due to low disease prevalence
- Focus on symptomatic care rather on holistic management
- Focus on prognosis rather on Quality of Life
- Lack of resources, infrastructure and skilled manpower
- Financial constraints

SOLUTIONS

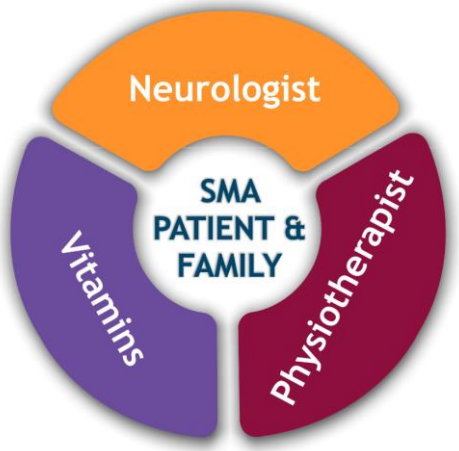
- Awareness activities
- Patient registry
- Education programs to enhance knowledge & skills
- Including Rare Disease in medical curriculum more elaborately
- Advocating importance of holistic care and multidisciplinary management for better quality of life with different stakeholders
- Policy level advocacy to include Rare Disease management and treatment in Government Healthcare program and health insurances

BENEFITS

- Multidisciplinary SMA clinics provide regular and comprehensive care
- Standardization of care protocol lowers cost and improve outcome
- Established centres and experienced clinicians to initiate Disease modifying therapy and clinical trials, studies and research

Standard of Care: Evolving Indian Scenario

2012 → **2017** → **2023**



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Multidisciplinary SMA Clinics in India

- CureSMA India started its first Multidisciplinary SMA Clinic in 2017 in collaboration with Peerless Hospital, Kolkata (Eastern India).

- Presently, in collaboration with various private/government hospitals, **seven** Multidisciplinary SMA Clinics are running across India in different geographical locations.

- Multidisciplinary Clinics and present SMA ecosystem in India is catering SMA individuals of India (including more than 1500 registered members of CureSMA India) and neighbouring countries like Bangladesh, Nepal, Bhutan, Sri Lanka and Pakistan (SAARC – South Asian Association for Regional Cooperation – countries).

CureSMA India SMA Task Force Team

- CureSMA India invited 21 renowned clinicians from various tertiary care hospitals across India to form the first SMA Task Force Team of India in 2020.
- The first SMA Task Force Team drafted COVID-19 guidelines for Indian SMA patients (available in CureSMA India website).
- The present CureSMA India Task Force includes 39 clinicians.
- This team is currently working on drafting Type-specific SMA guidelines for HCPs and caregivers.

*We sincerely '**Thank You**' for your time and consideration.*

Our families are truly looking up to you, for your support in our journey to 'Change & Save lives'!

Cure SMA Foundation of India

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