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Session Summary

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Executive Summary

Client-facing report -- SMA (Spinal Muscular Atrophy) Medical Affairs engagement landscape and non-promotional campaign plan (Brazil)

1) Objective and scope

Objective

Provide a fact-based view of the **current Medical Affairs engagement landscape for SMA in Brazil** and translate it into a **non-promotional campaign plan** focused on:

- **Newborn screening (NBS) gaps** and the operational reality of follow-up.
- **Regional disparities in diagnosis** and referral pathway friction.
- **Patient advocacy organisations** as campaign partners (governance-first).
- **Clinical expert and laboratory/reference-service networks** (KOL networks by institution/network, not by named individuals).

Scope boundaries (what this report does and does not do)

- **Included:** Disease/pathway education, screening literacy, operational closed-loop enablement, stakeholder engagement planning, governance and compliance context, and prioritized next steps.
- **Not included:** Product promotion, comparative therapy claims, brand messaging, or payer/price strategy. Where therapies are referenced, it is only to support the **public-health rationale** for early identification and appropriate referral.

2) Methodology (verification standard and source types)

2.1 Source types and evidence standard

Findings were developed using a tiered approach aligned to common Medical Affairs evidence expectations:

- **Tier A (Definitive):** Regulators and official government sources (e.g., ANVISA; Ministry of Health; official legal/portaria repositories).
- Used for: policy status, program descriptions, and regulatory approvals within jurisdiction.
- **Tier B (Substantive):** Peer-reviewed indexed literature (e.g., PubMed) and official registries where applicable.

- Used for: clinical rationale (e.g., value of presymptomatic identification), and implementation evidence.
- **Tier C (Contextual):** State government communications, reference-service reports, and credible institutional sources.
- Used for: state-level implementation signals, operational context, and local initiatives.

2.2 Cross-validation and claim discipline

- **Regulatory status claims** were anchored to regulator/government sources for the relevant jurisdiction.
- **Clinical-outcome framing** was anchored to peer-reviewed literature and expressed in appropriately hedged terms (e.g., “associated with” rather than “causes”).
- **State-by-state operational details** (what is included in panels, confirmatory routing, referral endpoints) were treated as **conditional** unless verified through state-level official sources or validated reference-service documentation.

2.3 Confidence grading used in this report

- **Verified:** Supported by regulator/government sources and/or peer-reviewed literature (as appropriate to claim type), or consistent multi-source confirmation.
- **Requires verification:** Plausible and contextually supported, but not confirmed with state-level authoritative sources or sufficiently current documentation.

3) Key findings by focus area (Brazil SMA)

3.1 Newborn screening and diagnosis pathway reality (Brazil)

Verified findings

- Brazil’s national newborn screening environment is **heterogeneous**, and the Ministry of Health has publicly described system challenges including **transport logistics, delays/absence in results delivery, and care gaps** in some regions.
- A formal federal framework exists for neonatal screening program organization and staged implementation updates (including newer GM/MS portaria changes).
- State-level expansion is **not uniform**; multiple states publicly communicate expanded heel-prick test panels and staged implementation priorities.

What this means (implications for Medical Affairs)

- The primary risk is not “lack of awareness that SMA exists,” but **pathway failure**:
- A screen or clinical suspicion does not reliably translate into **confirmatory testing + specialist referral + documented follow-through**.
- Therefore, the highest-yield Medical Affairs contribution is to enable a **closed-loop pathway** that reduces “lost-to-follow-up” and avoids “wrong door” referrals--without treatment promotion.

3.2 Regional disparities in diagnosis (operationally relevant) Verified findings

- National program-level communications describe **operational variability** and “care voids” as real barriers, implying that diagnosis and follow-up are not uniformly reliable across regions.
- State communications show that **expanded screening and operational capability differ materially by state** (some states explicitly prioritizing later stages of the national program).

Requires verification (but highly likely in practice)

- Lower-capacity regions are more likely to experience:
- Longer delays between first symptoms and specialist evaluation.
- Higher dependence on informal navigation (personal networks, advocacy groups).
- Greater variability in confirmatory testing logistics.

What this means (campaign design requirement)

- A Brazil-wide campaign should not ship a single “national pathway PDF.” It should implement:
- A **state overlay model** (state-specific inserts for confirmatory routing and referral endpoints).
- A strict **verification gate** before any overlay is externally distributed.

3.3 Patient advocacy organisations (as campaign partners) Verified findings

- Patient advocacy and rare disease coalitions are active in Brazil and serve as high-trust touchpoints for families, education, and navigation support.

Key partner reality (requires governance)

- Partnerships with advocacy organisations are high-impact but high-sensitivity:
- High privacy expectations.
- High reputational risk if perceived as promotional or steering.

What this means

- The campaign must be **governance-first**:
- Transparent partnership charters.
- Clear boundaries (education/navigation vs medical decision-making).
- Privacy-by-design (avoid identifiable data capture by default).
- A clear route for therapy-specific questions to compliant Medical Information channels.

3.4 Clinical expert and lab/reference-service networks (“KOL networks” by system nodes) Verified findings

- SMA screening and pathway initiatives in Brazil demonstrate the presence of:
- **Reference-service and laboratory capability** in multiple states.
- **Academic and specialist hubs** connected to screening and diagnostic workflow discussions.

Requires verification

- The detailed mapping of:
- Which labs act as confirmatory hubs by state.
- Which specialist services are the standard referral endpoints by state.
- Current capacity and turnaround times.

What this means

- The campaign should engage “KOL networks” as:
- **Operational validators** (workflow reliability, referral acceptance criteria).
- **Regional educators** (hub-to-spoke mentoring).
- **Quality improvers** (closed-loop QA huddles).

3.5 Disease awareness gaps (HCP and caregiver-facing) Verified findings

- The Ministry of Health’s framing of operational gaps implies that awareness alone is insufficient; structural delays can persist even when stakeholders understand the disease.

Requires verification

- Exact prevalence of misconceptions by region/specialty.

Most plausible high-yield awareness gaps (actionable, non-promotional)

- Among frontline clinicians:
- When to suspect SMA in symptom-first presentations.
- What “urgent referral” means and how to execute it locally.
- How to initiate confirmatory testing workflows.
- Among caregivers:
- What newborn screening does and does not cover in their state.
- What symptoms require urgent evaluation.
- Where to seek appropriate evaluation.

4) Recommended non-promotional Medical Affairs campaign architecture (working plan)

4.1 Guiding principles

1. **Closed-loop beats broadcast.** Optimize handoffs and follow-through, not just awareness volume.
2. **State overlays are mandatory.** Heterogeneity must be designed into the system.
3. **Governance-first with advocacy partners.** Trust and independence are strategic assets.
4. **Medical Information is the safety valve.** Route unsolicited treatment questions compliantly.
5. **Measure outputs credibly.** Avoid over-claiming outcomes without study designs.

4.2 Program structure (two-engine hybrid)

This plan combines two complementary engines:

Purpose: Ensure that screening/suspicion reliably becomes confirmatory testing and appropriate referral with documented follow-up. **Core deliverables (high level):**

- Closed-loop SOP with role clarity.
- Standard result/notification language and “do next” steps.
- Confirmatory routing and referral interface pack.
- Monthly closed-loop quality huddles (breakpoints and corrective actions).

Purpose: Reduce inequity by providing state-adaptable navigation and caregiver literacy through trusted partners. **Core deliverables (high level):**

- Partner charter, transparency, and privacy guardrails.
- Plain-language modular education kit.
- State-adaptable “where to go next” navigation template (with verified overlays).
- De-identified partner insight loop (journey breaks).

4.3 Minimum viable toolkit (what gets deployed)

A practical “minimum viable toolkit” for immediate deployment typically includes:

- **Frontline HCP toolkit:** fast-track algorithm + referral/testing pack + microlearning series.
- **Caregiver toolkit:** “next steps” handout with disclaimers and escalation guidance.
- **Lab/reference toolkit:** closed-loop SOP + reporting templates + QA cadence.
- **Partner toolkit:** charter + modular literacy + navigation template + insight intake.

Confidence note: Tool formats are well-established in Medical Affairs practice; however, final wording and workflows must be localized and verified.

4.4 State overlay approach and prioritization

Verified implementation signals exist in several states (examples include Minas Gerais and Espírito Santo), supporting a pragmatic rollout approach:

- **Tier 1 overlays (prove the model fast):** states with clearer public signals of expanded NBS/pilot activity and operational readiness.
- **Tier 2 overlays (equity priority):** states where public information suggests ongoing constraints or where navigation and symptom-first pathways may be especially important.
- **Tier 3 overlays:** broader national scale after a verification sprint.

Hard requirement: No external overlay distribution until the overlay evidence pack is complete (verification gate).

5) Confidence and data status summary (Verified vs Requires verification)

5.1 Snapshot table (what you can rely on now)

Topic	Verified	Requires verification
National program reality	Heterogeneity and operational issues described at the national level.	How these issues translate into local KPIs by state/region today.
State-level screening expansion signals	Multiple states publicly report expanded panels and staged priorities.	Exact current SMA inclusion status and routing details per state and how consistently implemented.
Pilot/initiative signal	Public description of a São Paulo pilot initiative exists.	Scope, coverage, and downstream follow-up performance metrics.
Advocacy partner ecosystem	Advocacy presence and activity are evident.	Partner readiness, governance maturity, and operational capacity by organisation.
Closed-loop pathway need	Strongly supported by national descriptions of delays and care gaps.	Measurable baseline follow-up completion rates (state-by-state).
Regulatory/clinical rationale	Regulator approvals exist; presymptomatic benefit supported in literature.	Brazil-specific outcome benchmarks linked to screening expansion (without dedicated studies).

6) Next steps (practical, time-boxed)

6.1 0-30 days: foundation and verification sprint design

1. **Agree non-promotional scope and governance language** with all internal stakeholders and partners.
2. **Define the state overlay template and evidence pack** (verification gate).
3. **Prioritize 3-5 pilot states** (Tier 1) based on verified public signals and operational feasibility.
4. **Stand up Medical Information readiness** (SRD topics, escalation rules, AE routing statement).

6.2 31-90 days: pilot execution

1. **Pilot the closed-loop SOP and reporting templates** with lab/reference-service leads in Tier 1 states.
2. **Deploy frontline HCP toolkit** (fast-track + referral pack + microlearning).
3. **Onboard 1-2 advocacy partners** under a signed charter and release the modular literacy toolkit.
4. **Launch the insight loop** (de-identified intake, monthly triage, update tickets).

6.3 3-12 months: scale and embed

1. Expand overlays to Tier 2 and Tier 3 states as verification completes.
2. Run a stable cadence:

- Monthly closed-loop QA huddles.
- Monthly MI trend review.
- Quarterly partner governance review and content refresh.

3. Prepare non-promotional scientific exchange outputs (implementation learnings).

7) Regulatory and compliance context (client-relevant)

Non-promotional guardrails (essential)

- All materials must be **disease/pathway education** and **operational enablement** only.
- Any therapy-specific questions must route through **Medical Information** (documented responses, audit trail).
- Avoid patient-identifiable data capture unless explicitly consented and governed; implement privacy-by-design principles consistent with Brazilian privacy expectations.

Operational compliance practices

- MLR review for external-facing assets (especially caregiver and advocacy partner materials).
- Controlled versioning and retirement of outdated pathway overlays.
- AE routing awareness for any channel likely to elicit treatment-related discussions.

8) Closing statement

A non-promotional SMA Medical Affairs campaign in Brazil is most likely to succeed when it is built as a **closed-loop operating system**--supported by state-verified overlays--and amplified via **governance-first advocacy partnerships** to address equity. The immediate priority is to validate state-specific routing and referral realities through a structured verification sprint, then scale with disciplined version control and Medical Information readiness.

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