



Corporate Presentation

Forward Looking Statements

Statements in this presentation that are not statements of historical or current fact constitute "forward-looking statements" within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, as amended, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and "forward-looking information" within the meaning of Canadian securities laws (collectively, "forward-looking statements"). Such forward-looking statements involve known and unknown risks, uncertainties, and other factors that could cause the actual results of Grace Therapeutics, Inc. (the "Company") to be materially different from historical results or from any future results expressed or implied by such forward-looking statements. In addition to statements which explicitly describe such risks and uncertainties, readers are urged to consider statements containing the terms "believes," "belief," "expects," "intends," "anticipates," "estimates," "potential," "should," "may," "will," "plans," "continue," "targeted" or other similar expressions to be uncertain and forward-looking. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this presentation. The forward-looking statements in this presentation, including, but not limited to, statements regarding the Company's belief that it can address the items related to chemistry, manufacturing, and controls ("CMC") and non-clinical information cited in the U.S. Food and Drug Administration's ("FDA") Complete Response Letter ("CRL") in a resubmission of its New Drug Application ("NDA") for GTx-104; the expected timing and outcome of the Type A meeting scheduled with the FDA related to the CRL; the Company's expectations that such Type A meeting with the FDA will clarify the path forward and determine next steps for GTx-104; the Company's plans to provide a regulatory update after the receipt of the official meeting minutes from such Type A meeting; expected cash runway; the potential exercise of outstanding warrants; the future prospects of the Company's GTx-104 drug candidate; GTx-104's potential to bring enhanced treatment options to patients suffering from aneurysmal subarachnoid hemorrhage ("aSAH"); GTx-104's potential to be administered to improve the management of hypotension in patients with aSAH; gastrointestinal intolerance and dosing consistency compared with oral administration; the ability of GTx-104 to achieve a pharmacokinetic and safety profile similar to oral formulations of nimodipine; GTx-104's potential to provide improved bioavailability and the potential for reduced use of rescue therapies; GTx-104's potential to achieve medical and pharmacoeconomic benefit over oral formulations of nimodipine; GTx-104's commercial prospects; the Company's pre-commercial launch strategy for GTx-104; the future prospects of the Company's GTx-102 drug candidate; GTx-102's potential to provide clinical benefits to decrease symptoms associated with Ataxia Telangiectasia; the timing and outcomes of a Phase 3 efficacy and safety trial for GTx-102; the timing of an NDA filing for GTx-102; the future prospects of the Company's GTx-101 drug candidate; GTx-101's potential to be administered to Postherpetic Neuralgia ("PHN") patients to treat severe nerve pain associated with the disease; the timing and outcomes of a Phase 3 efficacy and safety trial for GTx-101; the size of the addressable market for GTx-104 and GTx-102, and any future patent and other intellectual property filings made by the Company for new developments, are based upon Grace Therapeutics, Inc.'s current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, including, without limitation: (i) the outcome of any Type A meeting with the FDA related to GTx-104; (ii) the timing and success of any regulatory resubmission of the NDA for GTx-104; (iii) changes to regulatory requirements or regulatory pathways; (iv) the Company's ability to protect its intellectual property rights for its product candidates; and (v) legislative, regulatory, political and economic developments. The foregoing list of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors detailed in the "Special Note Regarding Forward-Looking Statements," "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of the Company's Annual Report on Form 10-K for the fiscal year ended March 31, 2026, and other documents that have been and will be filed by Grace Therapeutics, Inc. from time to time with the Securities and Exchange Commission and Canadian securities regulators. All forward-looking statements contained in this presentation speak only as of the date on which they were made. Grace Therapeutics undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by applicable securities laws.

Executive Summary

GTx-104 | aSAH



Nimodipine is the **SoC** and **clinically de-risked**; however, significant unmet needs remain with available oral formulations



GTx-104 – novel intravenous nimodipine – well positioned to **solve oral challenges** and potentially displace oral as SoC



Pivotal **Phase 3 STRIVE-ON** safety trial **met primary** endpoint; clinical **evidence of GTx-104 benefit vs oral capsules**



Potential to address a **severe rare disease** with **efficient commercial organization**; concentrated patient care



Orphan Drug Status with the potential for seven-year market exclusivity and **additional multi-layered IP protection**



Regulatory Update April 2026
FDA Complete Response Letter received;
Company intends to resubmit NDA following resolution of cited items

FDA Complete Response Letter (CRL) – Key Findings & Path Forward

- CRL driven by CMC data package completeness and manufacturing readiness
- **No clinical deficiencies identified**
- Type A Meeting Scheduled to Potentially Clarify Path to Resubmission

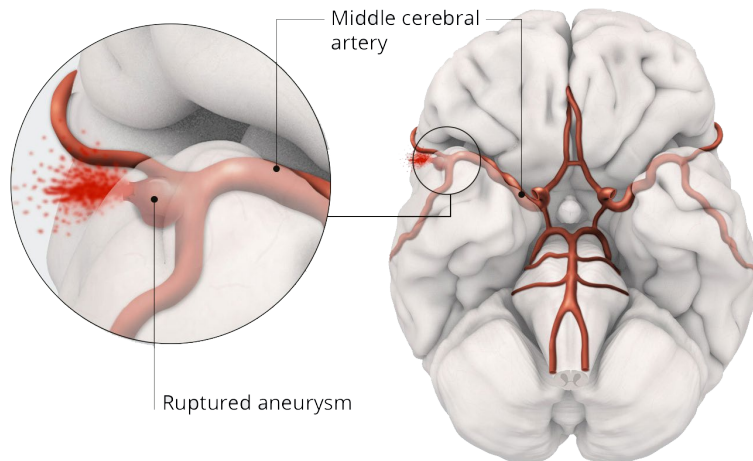
FDA Feedback	Company Assessment	Path Forward
<i>Leachable data</i> • Insufficient baseline and longitudinal data from intended commercial CDMO	• Dataset completeness related to commercial CDMO • Existing intermediate and long-term data generation already in place • <u>No indication of new safety signal</u>	• Generate baseline data from commercial site • Complete ongoing longitudinal dataset to support full shelf-life characterization • Align dataset and analytical approach with FDA expectations
<i>Nonclinical</i> • Unable to complete tox risk assessment without leachable dataset from CDMO • Additional assessment of excipient (Alcohol, USP) exposure requested. Maximum daily exposure of alcohol in drug product is within FDA inactive ingredient database	• Dependent on completion of leachable dataset • Alcohol concentration is less than 2% in infusion solution • No novel excipient used in drug product • <u>No standalone tox signal identified</u>	• Complete tox assessment based on updated leachable dataset from CDMO • Conduct targeted preclinical study for excipient duration of exposure as required
<i>Manufacturing Facility</i> • Deficiencies identified during cGMP inspection of CDMO	• Manufacturing compliance observations requiring remediation • <u>No product-specific quality issues identified</u>	• Ongoing remediation activities at CDMO • Potential reinspection prior to approval • Evaluating manufacturing alternatives to support supply and regulatory readiness

aSAH is a Rare and Severe Acute Brain Injury

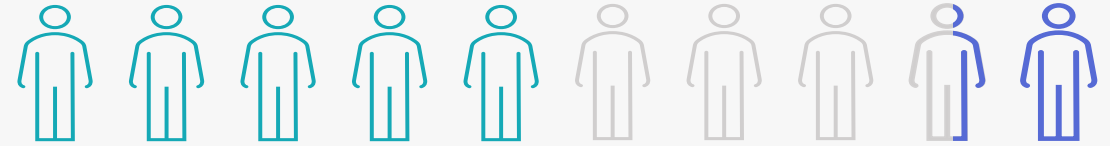


- aSAH results in bleeding over the surface of the brain in the space between the brain and skull
- Primary cause is rupture of an aneurysm
- Condition can occur quickly, immediate intervention is key to survival
- Patients require surgical intervention and oral nimodipine therapy

Subarachnoid Hemorrhage



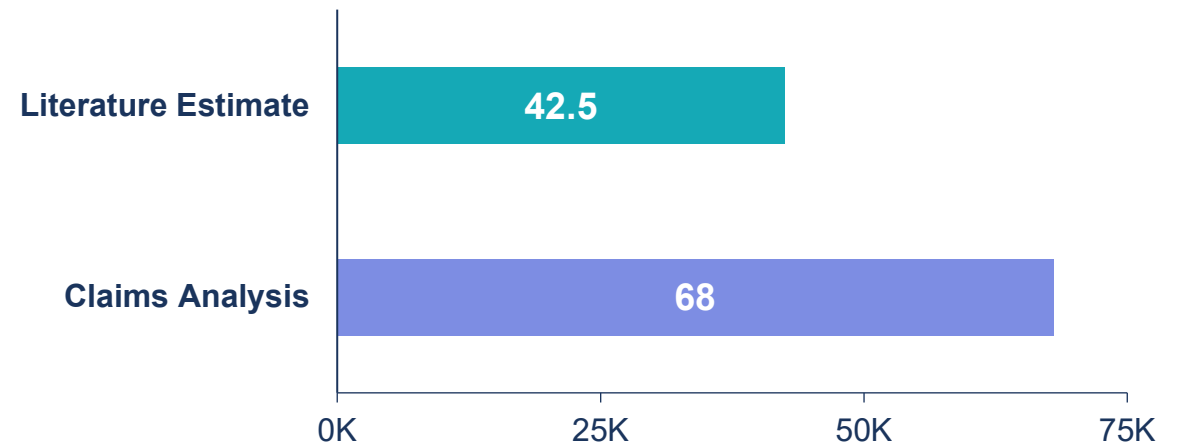
Occurs in Relatively Young Patients (~50% <60 yrs)



Significant Mortality (~10-15% before reaching hospital)

Est. Annual U.S. Hospital-Treated Patients (2023)

Hospital-treated aSAH may be as high as ~70k



Sources: ClearView Analysis (2025). Forian Claims Data. Fletcher Spaght market research; Becske T. (2018). Steven (2020).

Oral Nimodipine – The aSAH Standard of Care for >3 Decades

2023 AHA/ASA Guidelines

For the management of patients with aSAH

Recommended Use of Nimodipine for Management of Cerebral Vasospasm and DCI

"Early initiation of **enteral** nimodipine is beneficial in preventing DCI and improving functional outcomes"

Consistent Administration is Beneficial in Improving Functional Outcomes

"**Consistent administration** is suggested even in the setting of nimodipine-induced hypotension... However, if nimodipine causes significant BP variability, temporary stoppage may be necessary."

Recognition of the **Potential** for Differentiation of IV Nimodipine, with Additional Data

"Although studies of **intravenous** and intra-arterial nimodipine have been reported there are limited data to make any recommendation for these routes of nimodipine administration"

Nimodipine is the only approved therapy to improve neurological outcomes

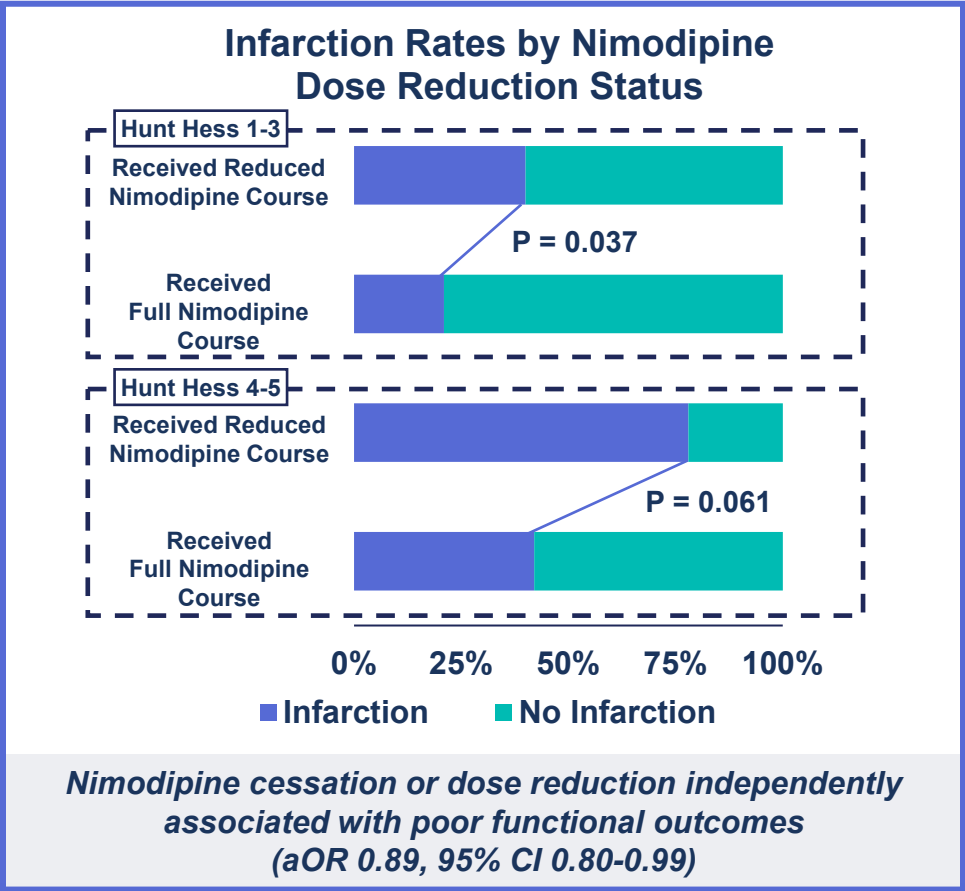
Limited use of off-label therapies due to The Joint Commission monitoring adherence to care guidelines

Sources: Hoh (2023). Hernandez-Duran (2019). Sandow (2016).

DCI: Delayed Cerebral Infarction

The Joint Commission is a hospital accreditation agency

Nimodipine – Consistent Drug Administration Drives Positive Patient Outcomes



Nimodipine is administered six times per day for up to 21 days
Limited use of off-label therapies due to Joint Commission monitoring adherence to care guidelines

Sources: Hoh (2023). Hernandez-Duran (2019). Sandow (2016).
aOR: adjusted odds ratio; CI: Confidence Interval

Substantial Shortcomings of Oral Nimodipine



Administration Challenges

- High dosing burden of 60mg (2 x 30mg capsules), 6 times per day
- 45% of patients receive nimodipine through nasogastric tube (NGT) – often via capsule extraction
- Capsule extraction and administration is labor intensive



Fatal Medication Errors

- Inadvertent parenteral injection can result in death or serious life-threatening AEs
- Highest risk with capsule extraction
- NYMALIZE (oral liquid) tempers the risk of error, but has tolerability challenges (e.g., severe diarrhea) due to solubility limitations of nimodipine

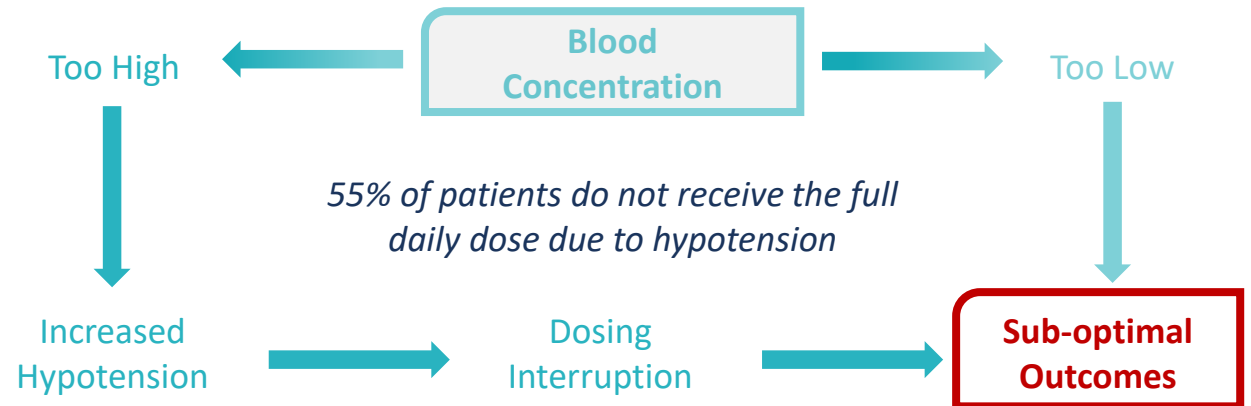


Sub-optimal Therapeutic Benefit with Oral Administration

High Pharmacokinetic Variability

- Inconsistent plasma concentration in both inter and intra subject
- High first-pass metabolism, leads to low bioavailability and frequent dosing
- Gastric motility issues and presence of food delay rate of absorption
- Potentially negligible concentration with NGT administration

Hypotension drives missed doses and diminished efficacy



GTx-104 is a Novel IV Nimodipine Designed to Overcome Oral Delivery Challenges Supported by Strong IP, Ph. III Trial Success

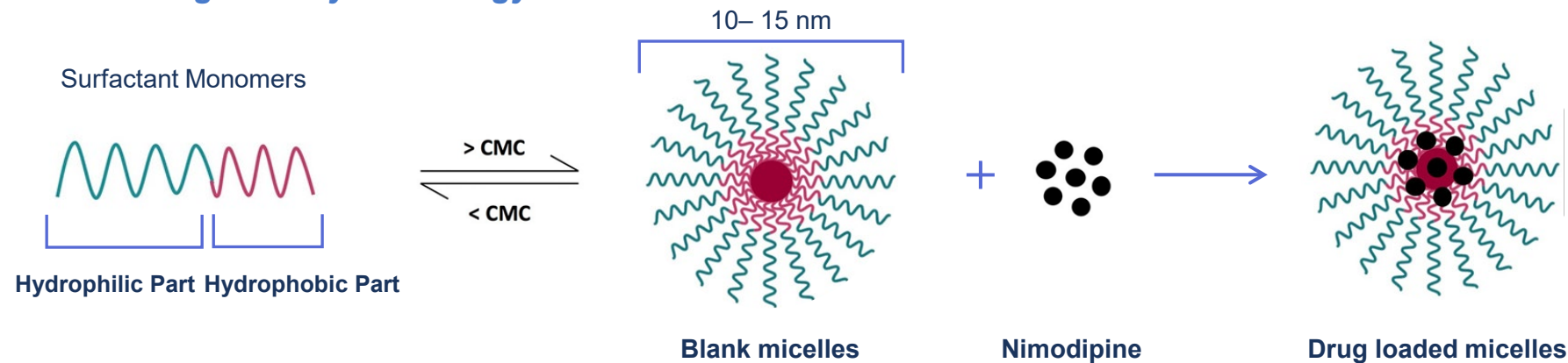
GTx-104

A novel intravenous nimodipine that is well positioned to **solve oral challenges** and potentially displace oral as SoC

Orphan Drug Status with the potential for seven-year market exclusivity and **additional multi-layered IP protection**

Pivotal **Phase 3 STRIVE-ON** safety trial **met primary** endpoint; clinical **evidence of GTx-104 benefit vs. oral capsules**

GTx-104 drug delivery technology



- Overcomes **solubility limitations** of nimodipine in current formulations
- Patented formulation uses non-ionic surfactant **micelles** as the drug carrier to **solubilize nimodipine**
- **Simple to prepare** in pharmacy, stable at room temperature

CMC: Critical micelle concentration.

Phase 1 Trial Established Scientific Bridge between GTx-104 and Oral Nimodipine

Trial met all primary and secondary endpoints; enabling the 505(b)2 regulatory pathway

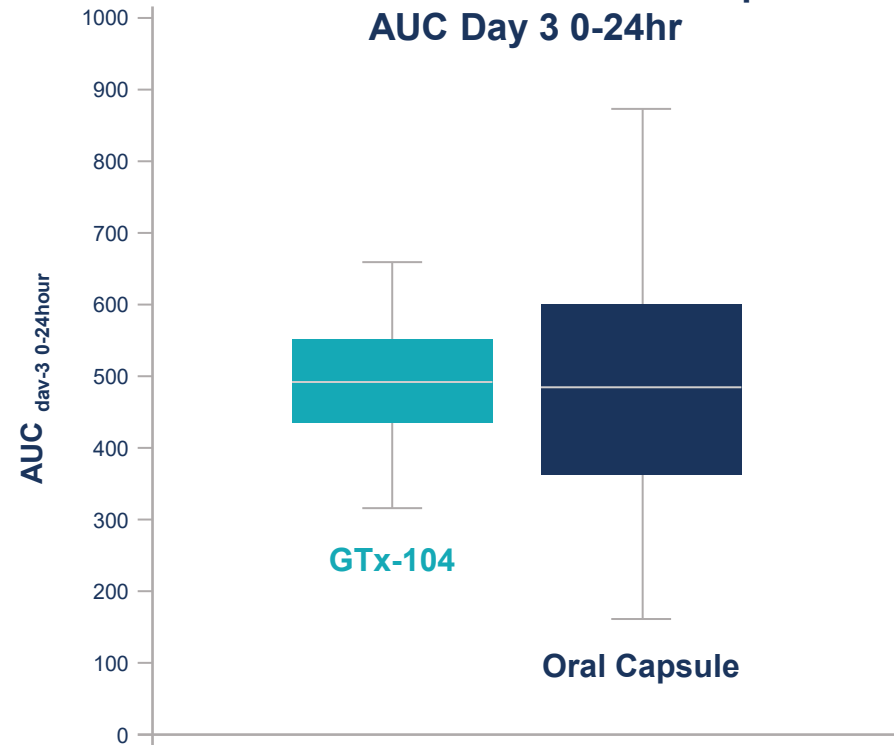


GTx-104

Consistent and *predictable*
plasma concentrations

Observed *lower dose* variability
relative to oral capsule

GTx-104 IV infusion vs Oral Capsule:
AUC Day 3 0-24hr



STRIVE-ON Phase 3 Trial



Trial complete and reported topline data in January 2025

STRIVE-ON (NCT05995405) is a ~100-patient prospective, open-label, randomized (1:1 ratio), parallel group trial of GTx-104 compared with nimodipine oral capsules in patients hospitalized for aSAH



Primary Endpoint
Incidence of subjects with at least one episode of clinically significant hypotension

STRIVE-ON Trial Data Support Potential Clinical, Pharmacoeconomic, and Dosing / Administration Benefits over Current SoC, Nimodipine Oral Capsules



CLINICAL

90-DAY OUTCOMES (MRS*)

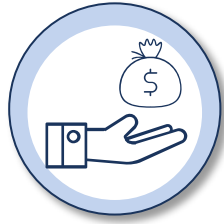
+29% relative increase in patients with good recovery at 90 days vs. oral capsules

HYPOTENSION EVENTS

-19% reduction from oral capsules

DOSE INTENSITY

54% vs. 8% with oral capsules receive >95% prescribed dose



PHARMACOECONOMIC

ICU DAYS

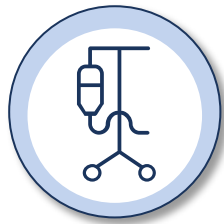
-1.5 days reduction from oral capsules

TIME ON VENTILATION

-5 days reduction from oral capsules

ICU READMISSION RATES

-48% reduction from oral capsules



DOSING & ADMIN.

PATIENT REST

No need to disrupt patient sleep every 4 hours

ADMINISTRATION

No feeding tube or swallowing of large pills required

TREATMENT PREP

No nimodipine capsule extraction and administration (laborious for staff)

* mRS: modified Rankin Score

Trial not statistically powered for hypothesis testing and comparisons between treatment arms

Demographics well-balanced, except higher proportion of most severe with worst prognosis (Grade V) in GTx-104

	GTx-104 (N = 50)	Nimodipine Oral Capsules (N = 52)
Age (mean)	55	56
Sex, n (%)		
Female	33 (66.0%)	33 (63.5%)
Male	17 (34.0%)	19 (36.5%)
Hunt & Hess Grade, n (%)		
I	10 (20%)	8 (15%)
II	15 (30%)	15 (29%)
III	15 (30%)	16 (31%)
IV	6 (12%)	12 (23%)
V	4 (8%)	1 (2%)

Primary Endpoint – Clinically Significant Hypotension



~19% relatively fewer patients with clinically significant hypotension in GTx-104

	GTx-104 (N = 50) n (%)	Nimodipine Oral Capsules (N = 52) n (%)
Clinically Significant Hypotension*	14 (28%)	18 (35%)

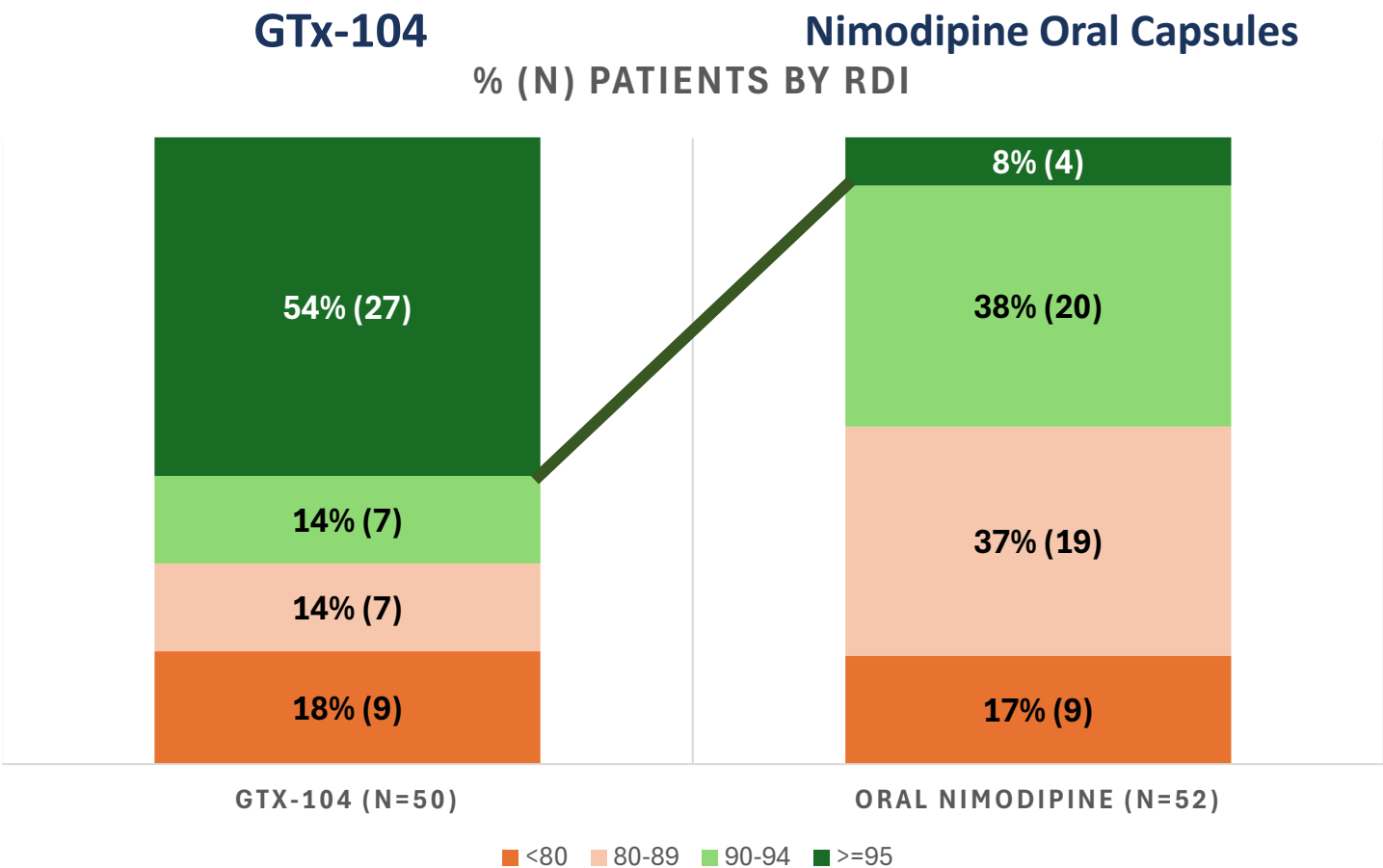
* Clinically significant hypotension: decrease in systolic BP > 20 mm Hg or diastolic BP > 10 mm Hg or systolic BP <= 100 confirmed by two consecutive readings within five minutes AND requiring medical intervention.

Trial not statistically powered for hypothesis testing and comparisons between treatment arms



Relative Dose Intensity (RDI)

54% of patients on GTx-104 had RDI of 95% or higher versus 8% on Nimodipine Oral Capsules



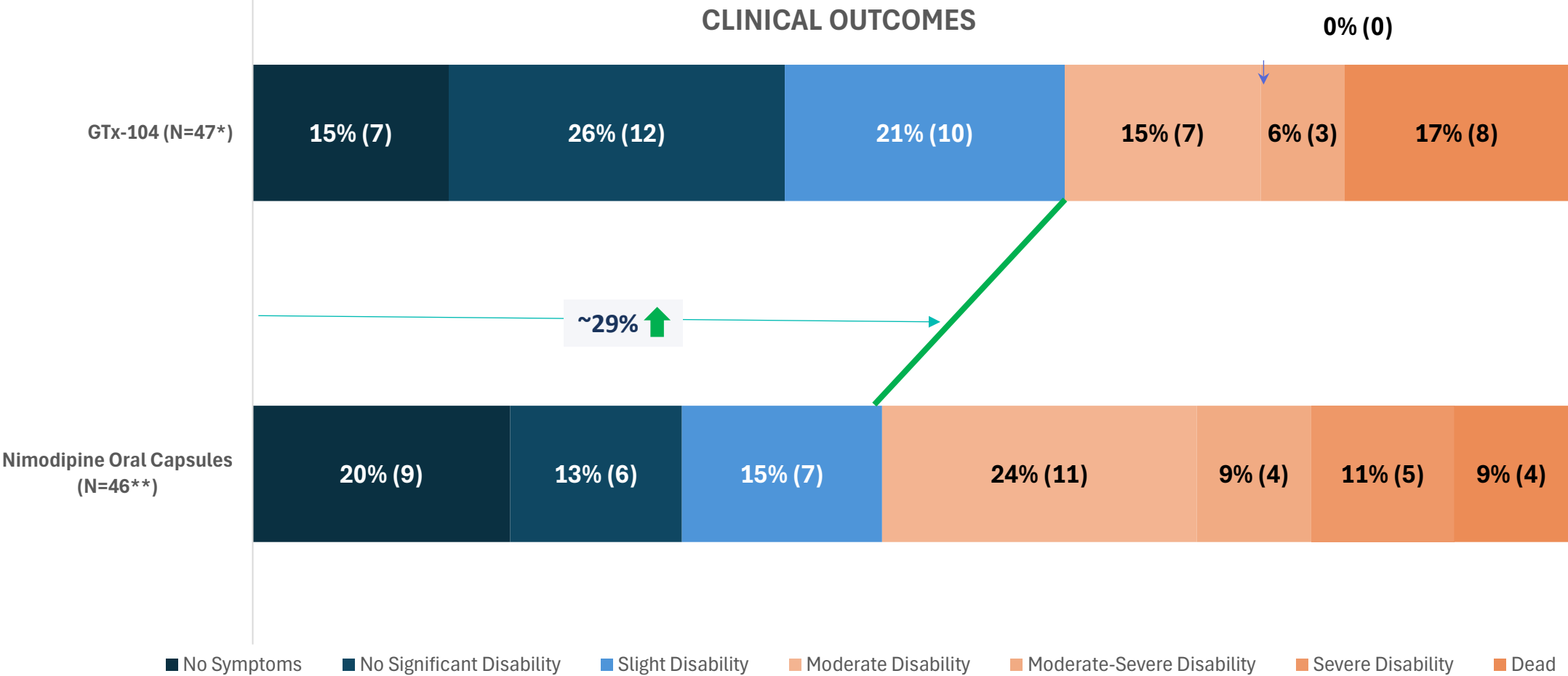
RDI: (total dose administered / total amount of expected dose) * 100

Trial not statistically powered for hypothesis testing and comparisons between treatment arms

Clinical Outcomes – mRS (day 90)



~29% relative increase in patients with good recovery in GTx-104



* 3 patients did not complete physician-conducted mRS at day-90. However, all 3 were confirmed alive at day-90
** 6 patients did not complete physician-conducted mRS at day-90. 5 were confirmed alive at day-90, and 1 survival status was unknown
Trial not statistically powered for hypothesis testing and comparisons between treatment arms



Clinical Outcomes – QoL (Quality of Life; day 90)

Patient-reported health scores favor GTx-104

QoL	GTx-104 (N = 38 ¹)	Nimodipine Oral Capsules (N = 40 ²)
Your Health Today Score mean (0 = being worst -> 100 = great)	75	70
Mobility, n (%) I have no or some problems I am confined to bed	38 (100%) 0	35 (88%) 5 (12%)
Self-Care, n (%) I have no or some problems I am unable to wash/dress	37 (97%) 1 (2.6%)	35 (88%) 5 (12%)
Usual Activities, n (%) I have no or some problems I am unable to perform	35 (92%) 3 (8%)	33 (84%) 7 (16%)
Pain/Discomfort, n (%) I have no or moderate pain I have extreme pain	36 (95%) 2 (5%)	38 (95%) 1 (2%)
Anxiety/Depression, n (%) I am not or moderately I am extremely	36 (95%) 2 (5%)	36 (90%) 3 (7%)

¹ GTx-104: patient did not complete survey (4), dead (8 – all due to underlying disease, none were GTx-104 related).

² Nimodipine Oral Capsules: patient did not complete survey (8), dead (4 – all due to underlying disease, none were related to oral capsules).
Oral also had 2 incomplete (pain, anxiety).

Overall safety was comparable between the two groups

Summary of Adverse Events (AEs) (entire study duration of 90 days)	GTx-104 (N = 50)	Nimodipine Oral Capsules (N = 52)
All AEs, n (%) # of events	44 (88%) 157	43 (83%) 193
All AEs, events per n	3.6	4.5
All SAEs ¹ , n (%) # of events	18 (36%) 34	25 (48%) 48
All SAEs, events per n	1.9	1.9
Treatment-Related SAEs, n (%) # of events ²	0	2 (4%) 2
Mortality ³ , n (%)	8 (16%)	4 (8%)
Cause of death ⁴ (n) All deaths were due to severity of underlying disease	No deaths due to GTx-104 — aSAH (5), ICH (1), rebleed (1), cardiac arrest (1)	No deaths due to Nimodipine Oral Capsules — aSAH (2), rebleed (1), cardiac arrest (1)

¹ A few include sepsis, deep vein thrombosis, ICH, hydrocephalus, cerebral infarction, urinary tract infection, C. difficile, systemic inflammatory response, acute kidney injury, as well as death

² Nimodipine Oral Capsules: bradycardia, vasospasm

³ Mortality rate is equivalent or lower than previous well-controlled clinical trials (Oral NIMOTOP NDA)

⁴ Based on investigator assessment

SAEs: Serious Adverse Events; ICH: Intracerebral Hemorrhage; DCI: Delayed Cerebral Hemorrhage

ICU Length of Stay (los), Mechanical Ventilator & Readmissions



1.5 fewer ICU days, 5 fewer ventilator days, and 48% relatively fewer ICU readmissions in GTx-104

	GTx-104 (N = 50)	Nimodipine Oral Capsules (N = 52)
ICU los, days Mean (SD)	16.4 (6.7)	17.9 (10.4)
Mechanical Ventilation days Mean (SD)	5.6 (5.7)	10.6 (13.9)
Hospital Readmissions*		
One readmission, n (%)	6 (12%)	7 (14%)
Two readmissions, n (%)	0	0
Three readmissions, n (%)	0	1 (2%)
ICU Readmissions		
One readmission, n (%)	2 (4%)	3 (6%)
Two readmissions, n (%)	0	1 (2%)

* Hospital Readmissions includes ICU readmissions. Readmissions were due to sequelae of aSAH e.g., UTI (urinary tract infection), DVT (deep vein thrombosis), Pneumonia, Seizures, Hydrocephalus, Cranioplasty.

SD: standard deviation

Trial not statistically powered for hypothesis testing and comparisons between treatment arms

Major patient resource utilization drivers in aSAH favor GTx-104

	GTx-104 (N = 50) n*			Nimodipine Oral Capsules (N = 52) n*		
	Day 1	Day 14	% change	Day 1	Day 14	% change
Mechanical Ventilation	14	1	-93%	12	7	-42%
External Ventricular Drain	32	10	-69%	35	17	-51%
Deep Sedation	5	1	-80%	8	5	-38%
Comatose	4	0	-100%	5	2	-60%

* Excludes patients that died before Day 14 for this analysis.

Trial not statistically powered for hypothesis testing and comparisons between treatment arms

Commercial Preparation



aSAH Market Opportunity

Addressable Patients

- **Literature**, typically limited to basal cistern aSAH (~80% of aSAH), suggests **~42.5K U.S. hospital-treated patients**
- **Claims analysis** suggests incidence of hospital-treated aSAH may be **as high as ~70K**

Most Critical Unmet Needs

- **~45%** of treated patients are **unconscious or dysphagic (nasogastric tube)**
- **>25%** of treated patients have **poor dose compliance / blood pressure control**

70% of aSAH Cases Result in Death or Permanent Disability

- **~50% of patients** who survive the initial month **remain permanently dependent** on a caregiver to maintain daily living
- **Hospitalization charges** can be up to **~\$530k** for an aSAH patient
- aSAH is among the **most highly reimbursed Diagnosis-Related Groups (DRGs)** in neuro ICU

Sources: ClearView Analysis (2025). Forian Claims Data. Becske T. (2018). Steven (2020). Hoh (2023). Etminan. JAMA Neurol. 2019; Fegin. The Lancet Neurology. 2009; Labovitz. Neuroepidemiology. 2006; Shea. Neurosurgery. 2007, Linn. Stroke. 1996; Anderson. Stroke. 2000; Daniere. J de Radiologie Diagnostique. 2015; Ingall. Stroke. 1989; Giordan et al. J Neurosurg. 2021; Rinkel et al. Lancet Neurol. 2011; Intl Study of Unruptured Intracranial Aneurysms Investigators. NEJM. 1998.

GTx-104 target product profile is seen as valuable for its improved tolerability, cost savings, and easy IV use – with broad formulary inclusion by P&T committee

Primary Market Research Insights (2Q 2025)



EFFICACY

Respondents emphasized that reduction in hypotension is meaningful, as it allows more patients to remain on therapy and **avoid dose-limiting side effects**

“... The pro of Product X is certainly the efficacy endpoint. The fact that there is a **reduction in hypotensive events**. That is a pretty significant, 19% reduction ...”
- Neurointensivist, Stanford University

“... The reduction in hypotensive events is meaningful. I could use it **for patients who can not take nimodipine due to hypotensive episodes** ...”
- Neurointensivist, Mount Sinai

“... I would prefer to **use Product X in every patient** because one of the biggest reasons to not continue nimodipine is because of hypotension ...”
- Neurointensivist, Atlantic Health System



PHARMACOECONOMIC

HCPs highlighted that even **modest reductions** in ICU or ventilator time can have a significant impact on hospital costs, suggesting potential to deliver **value beyond drug price**—particularly given the high-cost aSAH care settings

“... From an economic standpoint, fewer days in the ICU or on a ventilator certainly could justify the cost of the drug. **Even a reduction of a single day is relevant**. When it gets to be 2 or 3 days, then it's very impressive ...”
- Neurosurgeon, USC

“... A reduction in ventilator days is great for the patient in reducing their risk of infections and **benefitting their financial bottom line**. It's also good for hospital costs ...”
- Critical Care Specialist, Intermountain Health

“... Most hospitals are over capacity right now. Any reduction in ICU or ventilator days typically translates to shorter hospital days, which will **benefit hospitals overall in terms of costs and resources** ...”
- Neurointensivist, Boston Medical Center



ROA (route of admin)

Immediate usability without NG tube placement was seen as an advantage, **enabling earlier intervention**, especially in unstable or intubated patients where time sensitive dosing is key

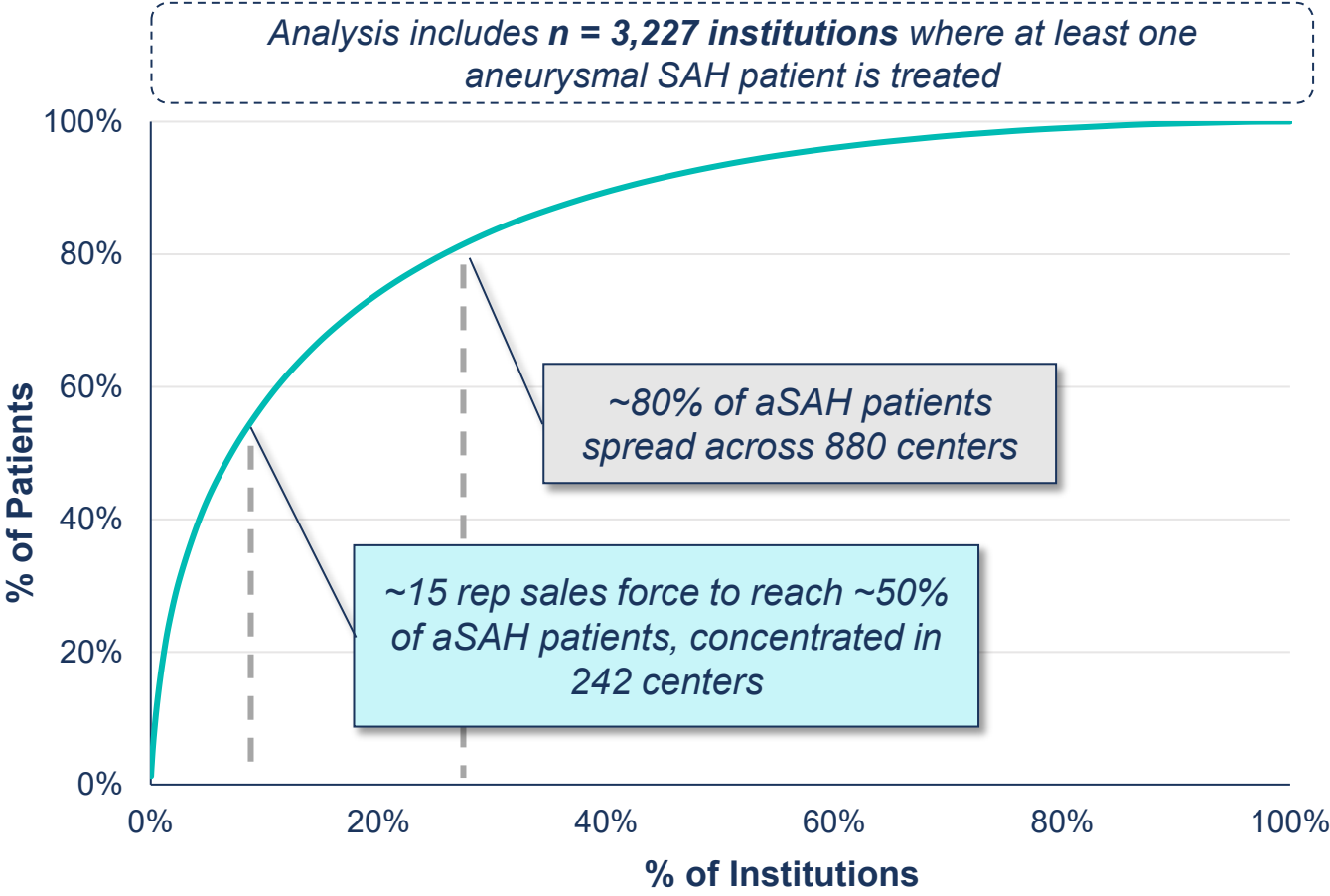
“... **I definitely prefer IVs for critically ill patients than oral**. You don't have to worry about placing down an NG tube ...”
- Neurosurgeon, Westchester Medical Center

“... Blood levels are more consistent from one dose to the other, which makes a whole lot of sense since it's IV. It's mostly maintaining a therapeutic level and being at the peak of concentration that is a major advantage of IV ...”
- Neurosurgeon, UCSF

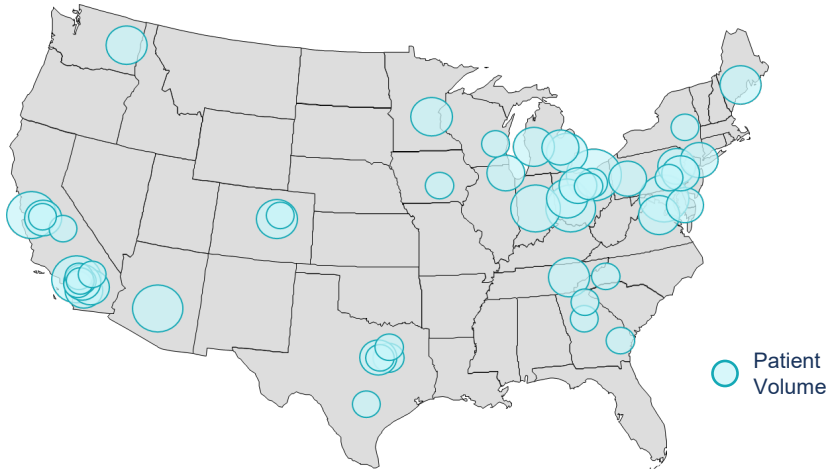
“... IV would be great because then **you don't have the NG tube anymore** or you don't need to rush for the NG tube because it's such a pain every time ...”
- Neurosurgeon, UCSF

Concentration of aSAH Care – Efficient Commercialization

Concentration of aSAH Patients



aSAH-Treating Institutions Concentration			
% aSAH Patients	% of Institutions	# of Institutions	Est. Sales Reps ¹
40%	~4%	146	~10
50%	~7%	242	~15
60%	~11%	380	~25



¹ Assumes each sales rep manages ~15 accounts.
Sources: ClearView Analysis (2025). Forian Claims Data.

Intellectual Property Portfolio

Multi-layered intellectual property protection strategy



GTx-104 received orphan drug status designation from the FDA

- Potential 7 years of marketing exclusivity in US upon NDA approval



US and international patent estate

- Consists primarily of formulation and method-of-use patents to extend exclusivity beyond what is granted through the orphan drug designation.
- Multiple patents granted worldwide, including six patents in the US
- Long patent shelf-life
 - First patent expiry 2037
 - Newest patent expiry 2043
- Continue building our patent portfolio by filing for patent protection on new developments

Capitalization

Grace Therapeutics, Inc. (GRCE) Cap Table (as of March 31, 2026)

Cash & Cash Equivalents Balance	USD \$17.0 M
Outstanding Common Stock	16,024,026
Debt	NONE
Stock options granted and outstanding	1,345,453
Total Fully Diluted Shares Outstanding ¹	23,033,731

Potential Gross Proceeds from Exercise of Outstanding Warrants

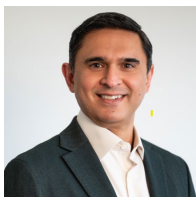
Feb-25 Private Placement ² : Potential Warrant Exercise Gross Proceeds	\$15.0 M
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¹ Includes Pre-Funded Warrants, Common Warrants, Outstanding Stock Options

² Represents warrants exercisable for 4,418,292 shares of common stock (or pre-funded warrants in lieu thereof) issued on February 11, 2025, with an aggregate exercise price of approximately \$15.0 million. The warrants are immediately exercisable at an exercise price of \$3.395 per share and will expire on the earlier of (i) the 60th day after the date the FDA approves the New Drug Application for GTx-104 and (ii) September 25, 2028.

Experienced Leadership Team

Management Team



Prashant Kohli
Chief Executive Officer



Loch Macdonald, MD, PhD
Chief Medical Officer



Amresh Kumar, PhD
VP Program Management



Robert J. DelAversano
Principal Financial Officer and
Principal Accounting Officer



Scientific Advisory Board

Andrew Ducruet, MD



Alex Choi, MD



W. Taylor Kimberly, MD, PhD



Alejandro A. Rabinstein, MD



Sherry H-Y Chou, MD



Deep aSAH Expertise in Research, Commercial, Drug & AHA Care Guidelines Development

Appendix (Deprioritized Programs)



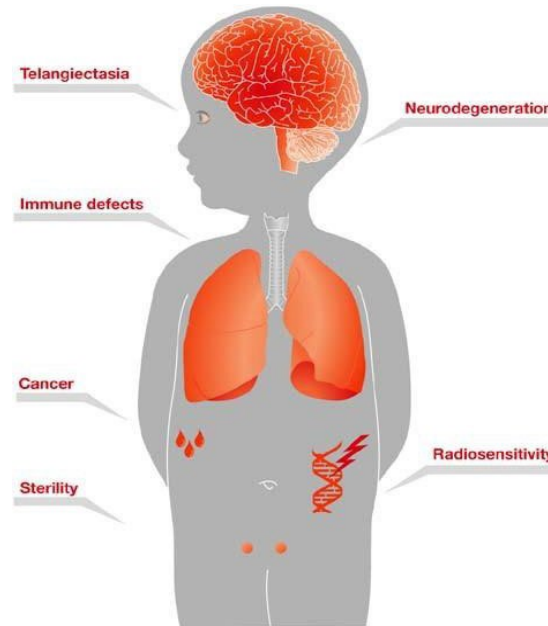
GTx-102 Program Overview & Regulatory Update

Ataxia-Telangiectasia

- Complex genetic neurodegenerative disorder diagnosed during infancy
- Inherited as an autosomal recessive trait, often affects more than one child in a family
- Average lifespan ~25 years
- Potential addressable market ~\$150 million

Unmet Need (No drugs approved)

- Treatment primarily directed toward control of symptoms
- Limited to speech, occupational and physical therapy
- Less than 20% of patients on any type of drug therapy for A-T symptoms



GTx-102

- Novel oral spray formulation of betamethasone intended to improve neurological symptoms of A-T patients
- Proof of concept supported by well-controlled Phase 1 trial with A-T patients
- PK bridging study topline results announced on 12/18/22 met all outcome measures

Regulatory

- FDA's written responses to EoP1 provides feedback on design of a single pivotal efficacy trial to support NDA
- Guidance includes primary endpoint scale and appropriate confirmatory evidence
- Plan to discuss with SAB potential trial design

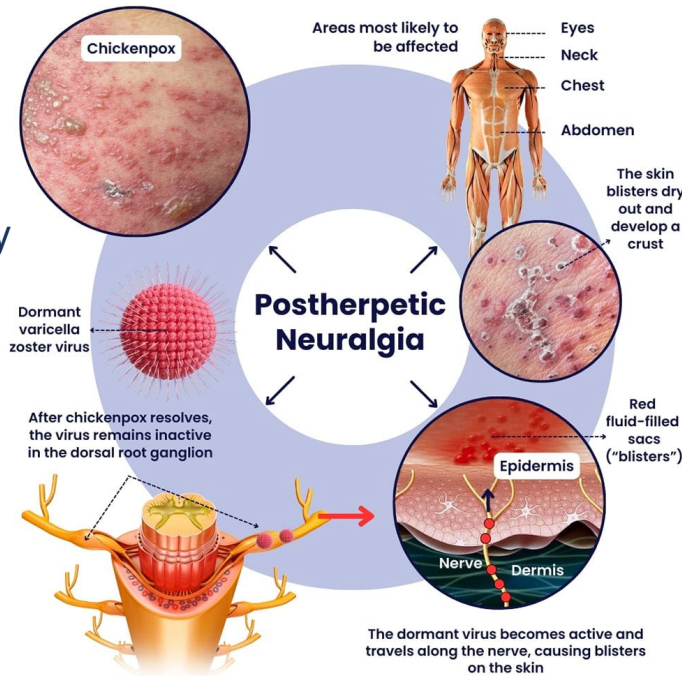
GTx-101 Program Overview

Postherpetic Neuralgia (rare disease)

- Caused by nerve damage from the herpes zoster virus which causes shingles
- Burning, painful, itchy, loss of feeling, sensitivity to touch or temperature, feeling worn out
- Symptoms can last for several years or may be permanent

Unmet Need

- Oral therapies (gabapentin, anticonvulsants, opioids) can have side effects and insufficient to manage pain in many cases
- Can be prone to abuse
- Lidocaine patches are hard to place, can cause skin irritation, are 12-hour on / off
- ~40% experience insufficient pain relief



GTx-101

- Non-narcotic, topical, bio-adhesive, transparent film-forming bupivacaine spray
- Biphasic drug release expected to provide immediate and continuous relief
- Potential Addressable market ~\$200m (PHN) to ~\$2.5b (lidocaine patch replacement)

Regulatory

- Completed Phase 1 (single dose) in 2022
- Met all primary outcome measures
- Clinical roadmap includes Phase 1 (multiple ascending dose) and Phase 2 (POC)