BB-031 in acute treatment: Safety, Tolerability, Pharmacokinetics (PK) and Pharmacodynamics (PD) in Acute Ischemic Stroke – The RAISE trial

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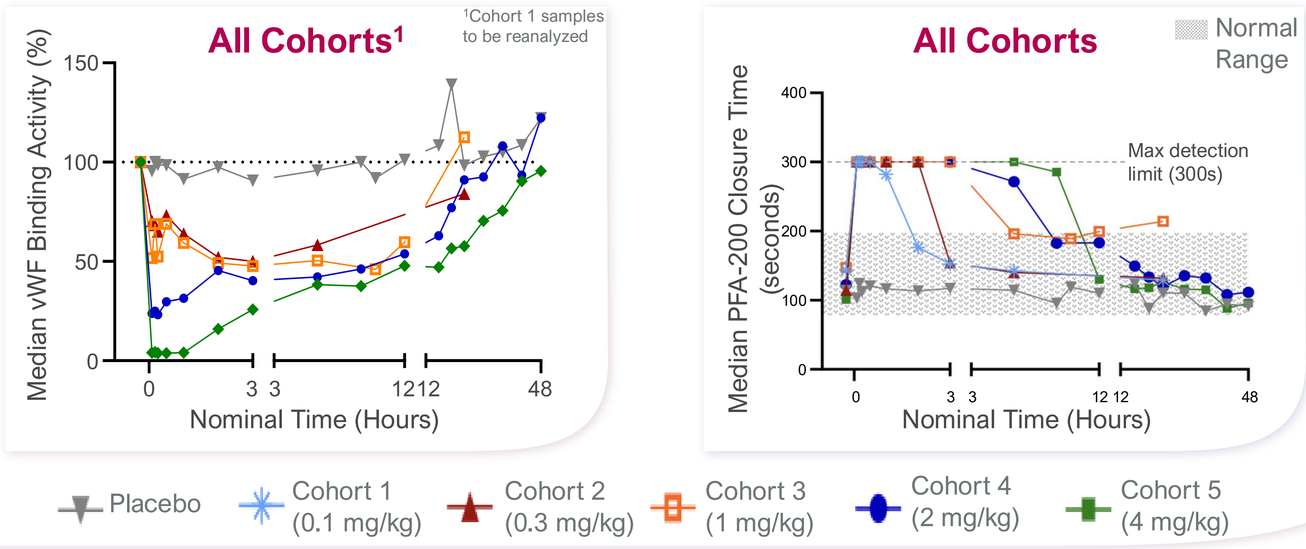
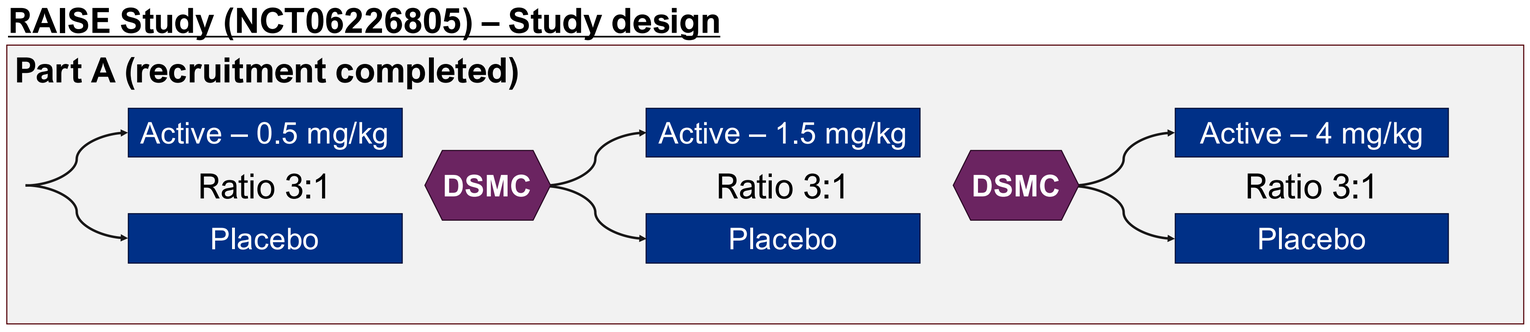
Introduction

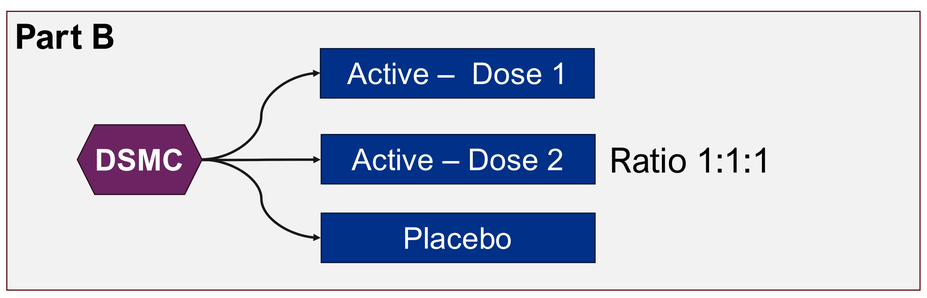
BB-031 is an RNA aptamer than rapidly binds to and inactivates circulating von Willebrand Factor (vWF) and is anticipated to impede and resolve thrombus formation and embolization. In nonclinical models, BB-031 prevents thrombosis *in-vivo* and effectively recanalizes arterial occlusions in small and large animals (Nimjee et al., 2012, 2019). Nonclinical toxicology studies were conducted in mice and canine models. Targeted inhibition of vWF by BB-031 increased recanalization and reperfusion, and reduced infarct volume in canine models of BAO and MCAO stroke. A first-in-human phase-1 study in healthy volunteers demonstrated that BB-031 was safe and inhibited vWF-binding in a dose-dependent manner.

Phase 1 results showed that dose-dependent vWF inhibition correlates with impact on platelet function



RAISE Study (NCT06226805) – Study design





RAISE Study – Patient Flow

Trial Design

- **Part A:** Single Ascending Dose
  - ~36 pts
  - *Part B dose selection based on Part A*
- **Part B:** 2 doses ~ 120 pts

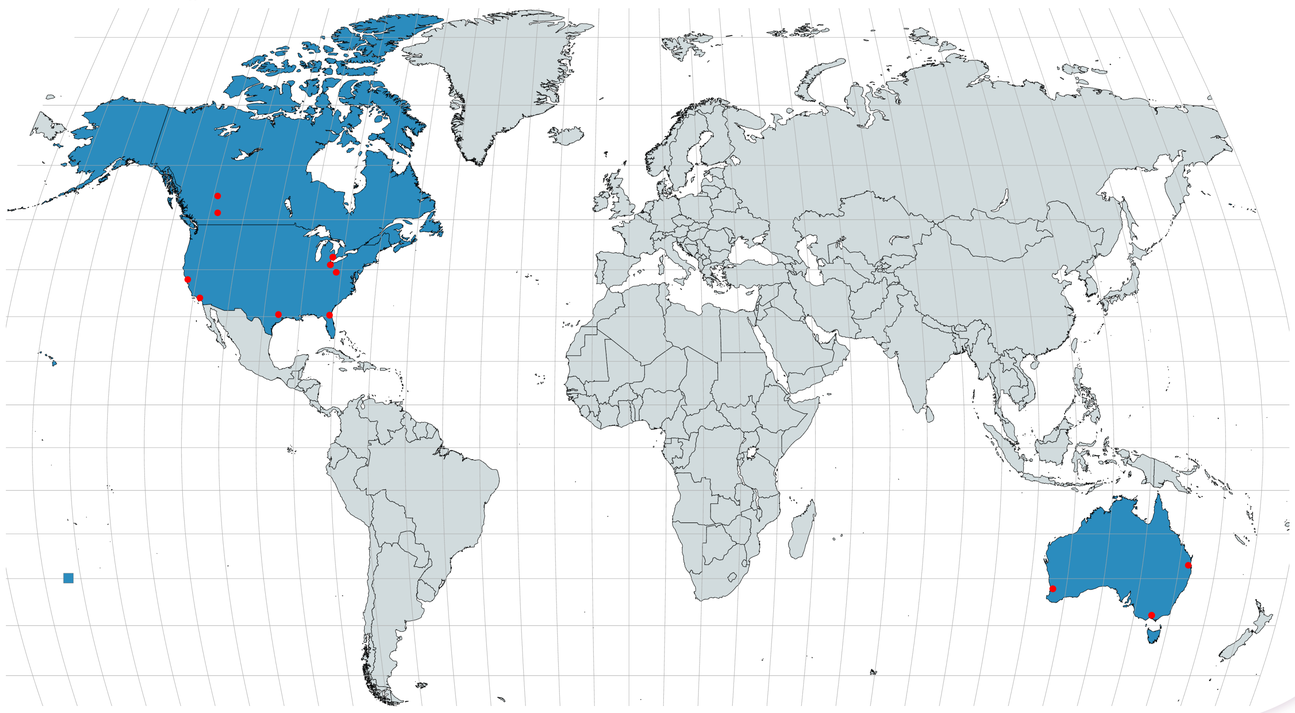
Screening for Enrollment

- 0-24 hours from symptom onset
- Rt-PA or TNK **not** administered
- Imaging: ANY-VO, and rule out large infarct, ensure homogenous population

Endpoints

- **Safety:**
  - sICH/non-symptomatic ICH proportion
  - Incidence and severity of Adverse Events
- **Secondary**
  - Recanalization via angiogram or CTA

RAISE Study – An International clinical trial



RAISE Study - Objectives

Primary

- Safety and tolerability of a single BB-031 dose in AIS patients

Secondary

- PK / PD of BB-031 in patients
- Recanalization within 24 hours after treatment

Exploratory

- Efficacy outcomes within 24 hours (NIHSS, mRS, Imaging)

RAISE Study – Main eligibility

Inclusion

- ✓ Anterior circulation intra-cranial occlusion
- ✓ 24 hours since Last Known Well
- ✓ Consent to participate

Exclusion

- Thrombolysis treatment (rt-PA, TNK)
- Large ischemic stroke volume (ASPECTS 0-5 or volume > 50 cc)
- Any ICH
- Treatment with anticoagulants or anti glycoprotein IIb/IIIa

Conclusion

The RAISE trial is the first patient trial of BB-031 evaluating vWF inhibition in the treatment of Acute Ischemic Stroke. Recruitment of the Part A cohorts is complete, dose selection for Part B is underway. Results from the RAISE trial will also inform planning of future efficacy trials. In addition to the RAISE trial, a new reversal agent, BB-025, a complementary rapid-acting reversal oligonucleotide capable of quickly neutralizing the pharmacological activity of BB-031, has been designed, with nonclinical testing underway.

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Basking Biosciences is a clinical-stage biopharmaceutical company, founded to solve the biggest need in acute thrombosis – for a rapid-onset, short-acting thrombolytic drug capable of reopening blocked arteries, and whose activity can be quickly reversed in the event of a bleeding complication.

https://baskingbiosciences.com/

Acknowledgments
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References
Carfora, A. *et al.* vWF targeted thrombolysis in canine basilar artery occlusion. *Front. Neurol.* **15**, 1436291 (2024).
Shea, S. M. *et al.* Dose-Dependent vWF Inhibition by Aptamer BB-031 Correlates with Thrombolysis in a Microfluidic Model of Arterial Occlusion. *Pharmaceuticals (Basel)* **15**, (2022).