synairgen

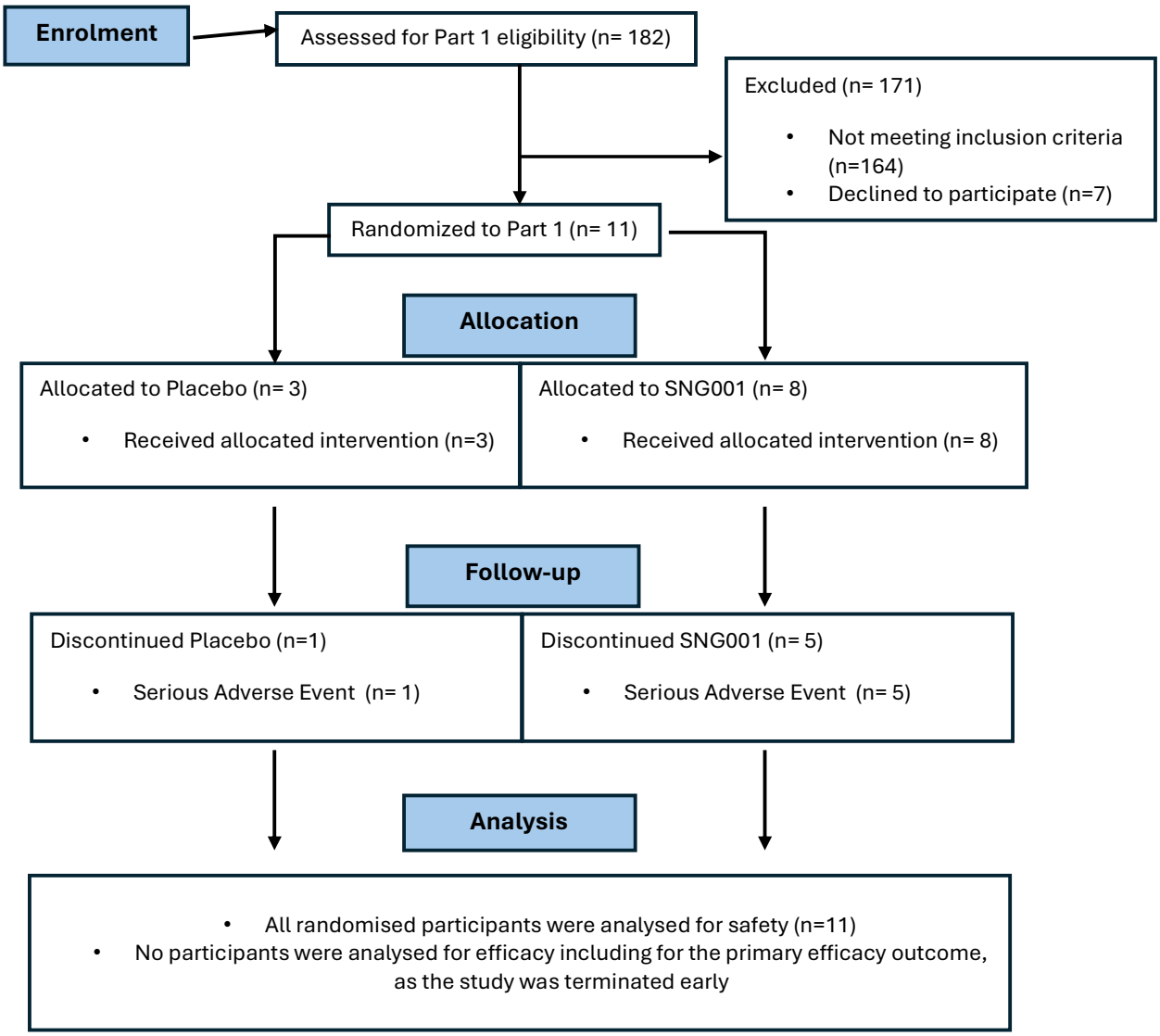
ISRCTN – SG021 Final study update

Section 1: Participant flow

During the study, participants were randomised to either SNG001 or placebo.

Participants to be randomised to active treatment in Part 1 of the study (defined as the safety cohort) received a low dose of SNG001. Part 1 was designed to include a total of 12 patients.

Participants to be randomised to active treatment in Part 2 of the study (defined as the efficacy cohort), were to receive a higher dose of SNG001. The study stopped prior to the completion of Part 1.



Section 2: Baseline characteristics

	Placebo + Standard of Care (SoC) N=3 n (%)	SNG001 + SoC N=8 n (%)
Sex at birth:		
Male	1 (33%)	4 (50%)
Female	2 (66%)	4 (50%)
Ethnicity		
Hispanic or Latino	1 (33%)	0
Not Hispanic or Latino	2 (66%)	8 (100%)
Race		
White	2 (66%)	4 (50%)
Black or African American	0	4 (50%)
Not reported	1 (33%)	0

Section 3: Outcome measures

The study was discontinued prematurely before Part 1 (the safety cohort) was completed. The low number of participants enrolled limits interpretation of the safety data collected. The data do not indicate any obvious safety signal associated with the administration of SNG001. No efficacy data were collected and no outcomes were measured as the trial was terminated early.

Section 4: Adverse events

The proportion of participants with treatment emergent adverse events (TEAEs) reported during the trial was higher in the SNG001 arm (n=8 [100%]) than in the placebo arm (n=2 [66.7%]), as was the proportion of participants with serious TEAEs (SNG001: n=7 [87.5%]; placebo: n=2 [66.7%]). However, the low numbers of participants enrolled limits the interpretation of these results. One participant in the SNG001 arm experienced a serious TEAE considered possibly related to study treatment by the investigator (alanine aminotransferase increased). Four out of 8 participants (50%) in the SNG001 arm died, compared to 1 out of 3 participants (33.3%) in the placebo arm. None of the deaths were considered to be treatment related. One participant in the SNG001 arm discontinued study treatment due to a TEAE (hypoxia).

Adverse Event Summary (Safety Cohort)

	Placebo + SoC N=3 n (%)	**SNG001 + SoC N=8 n (%)**
Any Treatment emergent adverse event (TEAE)	2 (66.7%)	8 (100%)
Mild	2 (66.7%)	2 (25.0%)
Moderate	2 (66.7%)	2 (25.0%)
Severe	2 (66.7%)	7 (87.5%)
Serious TEAE	2 (66.7%)	7 (87.5%)
Serious related TEAE	0	1 (12.5%)
Fatal TEAE	1 (33.3%)	4 (50.0%)
Related fatal TEAE	0	0
TEAE related to study treatment	0	1 (12.5%)
TEAE leading to discontinuation of study treatment	0	1 (12.5%)
TEAE of note	0	0

n: Number of participants with an event, N: Total number of participants dosed, SoC: Standard of care, TEAE: Treatment emergent adverse event.
Percentages are calculated using the total number of participants in each treatment group (N).
A TEAE is defined as any event beginning or worsening or becoming serious on or after the first dose of study drug in the respective period.