GOTHAM Basic Results Summary

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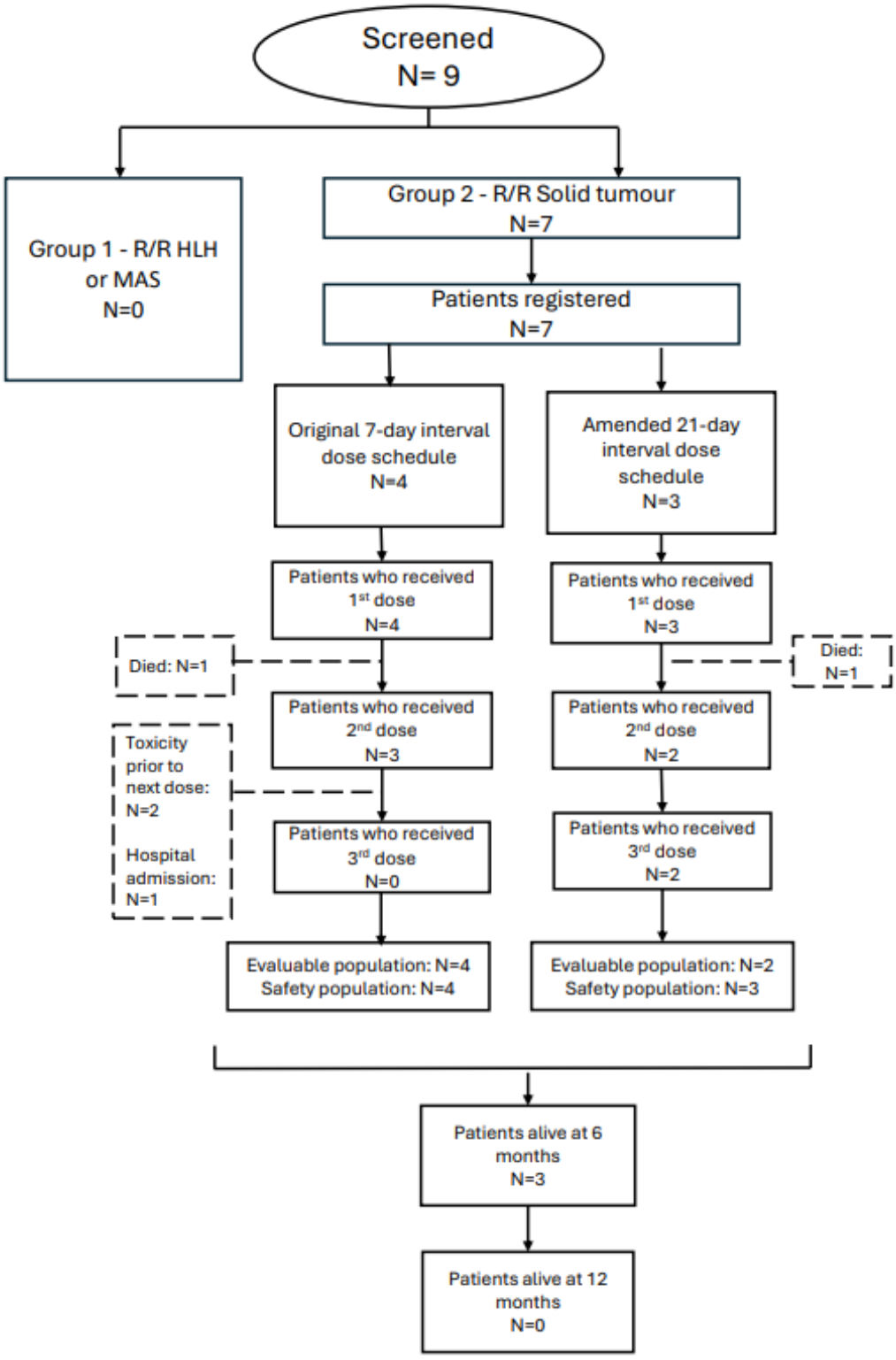
1. Participant Flow



2. Baseline Characteristics

		Result
Gender	Female	2 (29)
	Male	5 (71)
Age (years)	Median (IQR)[n]	65 (55, 67) [7]
Disease	R/R Solid cancer (Group 2)	7 (100)
Disease type	Localised	1 (14)
	Metastatic	6 (86)
Original tumour type	Ewing's Sarcoma	1 (14)
	Adenocarcinoma	2 (29)
	Adenocarcinoma of Sigmoid Colon	1 (14)
	Adenocarcinoma sigmoid colon	1 (14)
	Rectal sigmoid tumour - adenocarcinoma	1 (14)
	Sigmoid colon	1 (14)
Months since diagnosis	Median (IQR)[n]	37.5 (27.7, 40) [7]
Relapse confirmation	Radiologically	7 (100)
Months since last treatment failure	Median (IQR)[n]	3.3 (1.3, 5.3) [7]
Haemoglobin (g/L)	Median (IQR)[n]	97 (91, 136) [7]
White blood cells ($x10^9$/L)	Median (IQR)[n]	10 (7.1, 11.9) [7]
Neutrophils ($x10^9$/L)	Median (IQR)[n]	6.5 (4.1, 8.5) [7]
Platelets ($x10^9$/L)	Median (IQR)[n]	306 (185, 588) [7]
ALT (U/L)	Median (IQR)[n]	45 (14, 79) [7]
AST (U/L)	Median (IQR)[n]	63.5 (39, 88) [2]
Total Bilirubin (umol/L)	Median (IQR)[n]	8 (7, 17) [7]

Table shows N (%) unless otherwise stated

3. Outcome Measures

Primary Outcome Measure:	
CD33+ cell count in the blood samples of patients collected on day 1, day 8, day 15, day 22, 43, 50 and 57	**Relative Change in CD33+ count:** Relative change is defined as the final value of CD33+ count (ideally taken on day 57) minus the initial value of CD33+ count (taken on day 1 of treatment) all divided by the initial value. A negative value of relative change therefore suggests a reduction in CD33+ cell count. 2 out of 6 patients had a recorded value at day 57 which was used in the calculation. Of the remaining 4 patients, 3 had their final CD33+ value recorded on day 29 and 1 on day 8. Due to this a sensitivity analysis, including only patients who had a day 57 value recorded has also been presented. Given the low number of patients

within the trial, medians and interquartile ranges are provided for relative change instead of mean (and standard deviations).

CD33+ relative change:

	Median (IQR)[n]
Relative Change	0.17 (-0.77, 0.5) [6]
Relative Change – Sensitivity Analysis	-0.14 (-0.77, 0.5) [2]

Greatest absolute change in CD33+ Count:

Greatest absolute change is defined as the largest absolute value difference in CD33+ count from day 1 of treatment to any other subsequent time point. A negative value of greatest absolute change therefore suggests a reduction in CD33+ cell count.

CD33+ greatest absolute change:

	Median (IQR)[n]
Greatest absolute change	-418.8 (-699.4, -353.5) [6]

Time to reach lowest value of CD33+ count:

Time to reach lowest value is defined as the number of days to the day the lowest value of CD33+ is measured. As measurements are taken on predefined days (1, 8, 15, 22, 29, 43, 50, 57) the time to reach the lowest value is either (0, 7, 14, 21, 18, 42, 49 or 56 days) for each patient. Additionally, it is also possible for patients to deviate from the treatment plan, as such these times will also be calculated as the difference between registration date and the date the was taken, this will be referred to as the observed time to lowest value. The tables below show a tabulation of the time point that the lowest value was presented at and a summary of the observed time to the lowest value presented as a median with interquartile range.

Time to lowest value:

Time to reach lowest value (days)	N (%)
7	3 (50)
14	3 (50)

Observed time to lowest value:

	<table><tr><td></td><td>Median (IQR)[n]</td></tr><tr><td>Observed time to reach lowest value</td><td>12.5 (8, 15) [6]</td></tr></table>
Secondary Outcome Measures:	
Overall Survival time - defined as the time from the date of entry into the trial to the date of death	Overall survival time is defined as time from date of entry into the trial to the date of death. Patients who are alive at the time of analysis will be censored at their date last known to be alive. The tables below show the estimates of overall survival at 3, 6, 9, & 12 months and the median overall survival. Overall survival time point estimates: <table><tr><td>Time Point</td><td>OS (95% CI) (%)</td></tr><tr><td>3 months</td><td>83.3 (27.3, 97.5)</td></tr><tr><td>6 months</td><td>50.0 (11.1, 80.4)</td></tr><tr><td>9 months</td><td>16.7 (0.8, 51.7)</td></tr><tr><td>12 months</td><td>16.7 (0.8, 51.7)</td></tr></table> Median overall survival: <table><tr><td></td><td>Months</td></tr><tr><td>Median (95% CI)</td><td>6.53 (0.46, .)</td></tr></table> Appendix #1 shows the Kaplan Meier plot of Overall Survival.
Progression-free survival (PFS) time (Group 2 only) - defined as the time from the date of entry into the trial to the date of disease progression	Progression free survival time is defined as time from date of entry into the trial to the first of progression. Patients who are alive and progression free at the time of analysis will be censored at their date last known to be alive. The tables below show the estimates of progression free survival at 3, 6, 9, & 12 months and the median progression free survival. Progression free survival time point estimates: <table><tr><td>Time point</td><td>PFS (95% CI) (%)</td></tr><tr><td>3 months</td><td>33.3 (4.6, 67.6)</td></tr><tr><td>6 months</td><td>16.7 (0.8, 51.7)</td></tr><tr><td>9 months</td><td>16.7 (0.8, 51.7)</td></tr><tr><td>12 months</td><td>16.7 (0.8, 51.7)</td></tr></table> Median Progression Free Survival: <table><tr><td></td><td>Months</td></tr><tr><td>Median (95% CI)</td><td>2.07 (0.13, .)</td></tr></table> Appendix #2 shows the Kaplan Meier plot of Progression Free Survival.

4. Adverse Events

A total of 15 adverse events have been reported in 5 patients. The table below shows all adverse events by grade. Of the four patients treated on the original dose schedule, 3 patients experienced 8 adverse events (2.67 events per patient). Of the 3 patients treated at the updated dose schedule, 2 patients experienced 7 adverse events (3.5 events per patient).

All adverse events by grade:

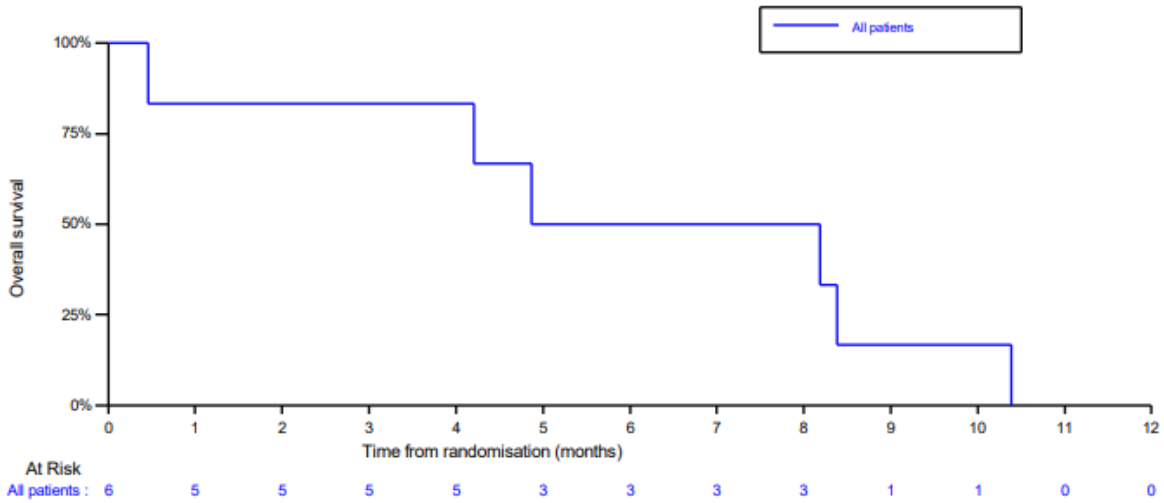
Toxicity	Events (patients)
Abdominal Pain	3 (2)
Anemia	1 (1)
Back Pain	1 (1)
Biliary tract infection	1 (1)
Dyspnea	1 (1)
Fatigue	1 (1)
Febrile Neutropenia	1 (1)
Infections and infestations – Other, specify	1 (1)
Neutrophil count decreased	3 (3)
Respiratory failure	1 (1)
White blood cell decreased	1 (1)
Total	15 (5)

5. Serious Adverse Events

A total of 5 SAEs have been reported in three patients. Details of all events are reported in the table below. Three of these events occurred in two patients treated on the original dose schedule and one occurred in a patient on the updated dose schedule.

Toxicity	Events (patients)
Abdominal Pain	3 (2)
Febrile Neutropenia	1 (1)
Infections and Infestations – Other Infection – Chest / Infectious Pneumonia	1 (1)
Total	5 (3)

Appendix #1 – Kaplan Meier Plot of Overall Survival



Appendix #2: Kaplan Meier Plot of Progression Free Survival