Results of a Phase 1 Trial of GDA-201, Nicotinamide-Expanded Allogeneic Natural Killer (NK) Cells in Patients with Refractory Non-Hodgkin Lymphoma and Multiple Myeloma

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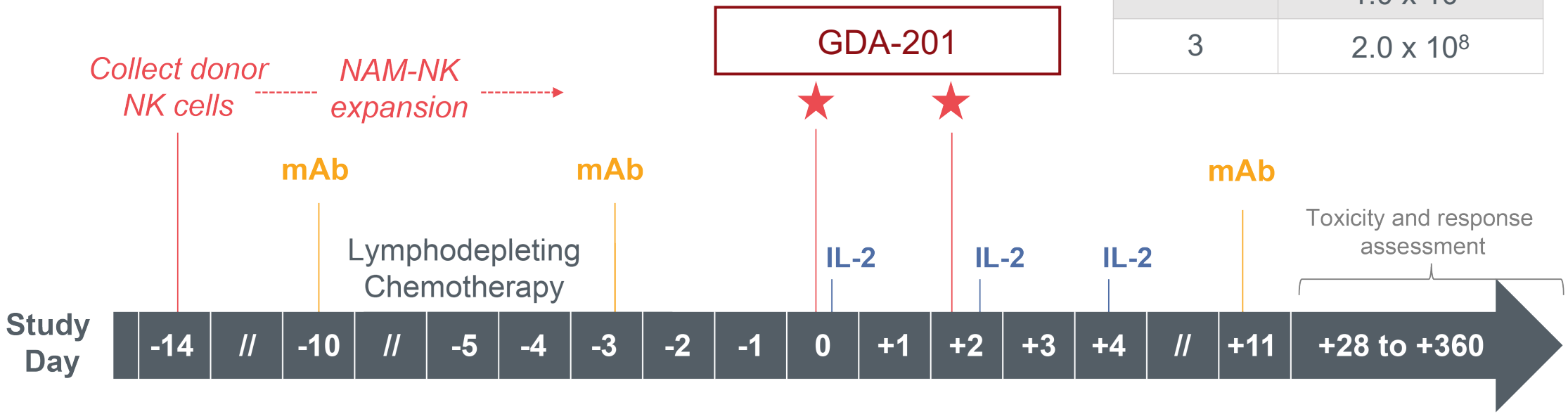
Phase 1 GDA-201 Study Schema and Dose Cohorts

Monoclonal antibodies (mAb): Rituximab (375 mg/m2 IV) for B-cell lymphoma or Elotuzumab (10 mg/kg IV) for multiple myeloma,

Lymphodepleting chemotherapy: Cyclophosphamide (400 mg/m2 IV) x 3d and fludarabine (30 mg/m2 /d IV x 3d)

IL-2: 6 million units sc

GDA-201 Cohort	Target TNC Dose (cells / kg)
1	2.0×10^7
2	1.0×10^8
3	2.0×10^8



ClinicalTrials.gov Identifier NCT03019666

Phase 1 GDA-201 Study: Patient Demographics

Patient and Disease Characteristics	Total N = 35	NHL cohort n=19
Age [median (range)]	61 (46-83)	60 (46-83)
Gender: male/female	21/14	11/8
Dg: MM NHL	16 19	19
Diffuse large B cell lymphoma	-	8
Follicular lymphoma	-	10
MCL	-	1
Disease status		
Relapsed	28 (80%)	16 (89%)
Refractory	7 (20%)	3 (11%)
Stage III-IV (NHL only)	-	15 (82%)
Number of lines of Therapies median (range)	4.5 (1-10)	3 (1-8)
Prior autologous transplant	16 (47%)	3 (17%)
Prior allogeneic transplant	1 (3%)	1 (5.6%)
KPS 80 or less	16 (47%)	8 (45%)
GDA-201 cell dose median in 10^{*7} /kg (range)	**14.3 (2.0-26.0)**	**10.2 (2.0-26.0)**

Dose Limiting Toxicities

- No dose limiting toxicities.
- No GVHD
- No neurotoxicity events
- No marrow aplasia.
- One patient with MM died Day 27 due to E coli sepsis; reported as grade 3 CRS but not confirmed

Grade 3-5 Adverse Events (N=35)

- **Adverse events mostly attributed to lymphodepleting chemotherapy**
- **Most common adverse events were decreased neutrophil count, febrile neutropenia, anemia and low platelet counts**

	Severity			
Event	Grade 3	Grade 4	Grade 5	Total
Hematologic	9	19	0	28
Anemia	3			3
Febrile neutropenia	4	3		7
Neutrophil count decreased	2	10		12
Platelet count decreased		3		3
White blood cell decreased		3		3
Cardiac and Vascular	8	2	0	10
Arythmia	3	1		4
Hypertension	4			4
Hypotension	1	1		2
Pulmonary	6	1	**0**	8
Dyspnea/Tachypnea	3			3
Hypoxia	2			2
Pneumonia		1		2
Pulmonary Edema	1	1		2
Infectious/Immune	3	0	**1**	4
Cytokine release syndrome	1			1
Sepsis			1	1
Upper respiratory infection	2			2
Other	18	2	0	20
Fever	2			2
Pain	4			4
Electrolyte abnormality	5			5
Generalized weakness	2			2
Confusion	1			1
Rash	1			1

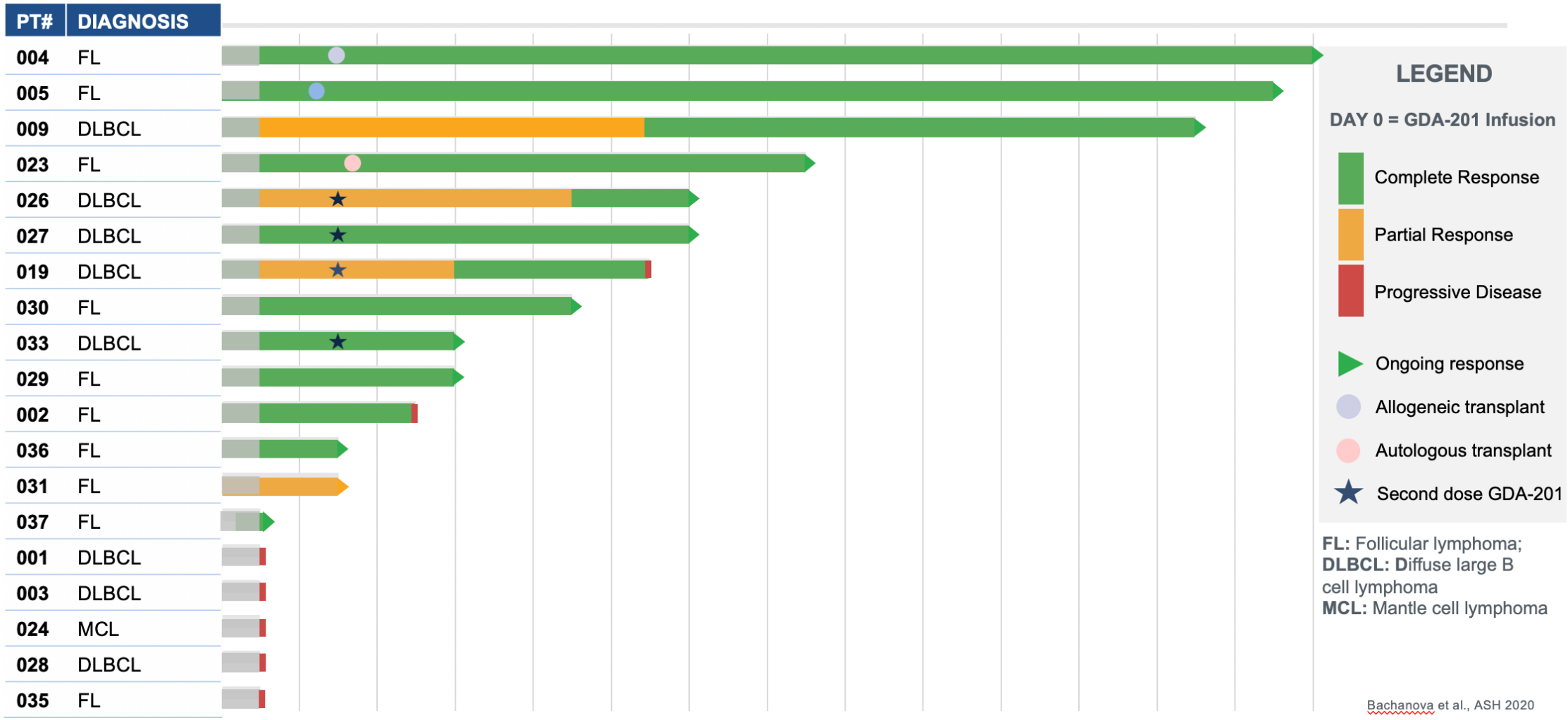
Response Rates

19 patients with NHL treated, median duration of follow-up 10 months

- 13 CR
- 1 PR
- 5 PD
- ORR: 74%
- CR rate: 68%

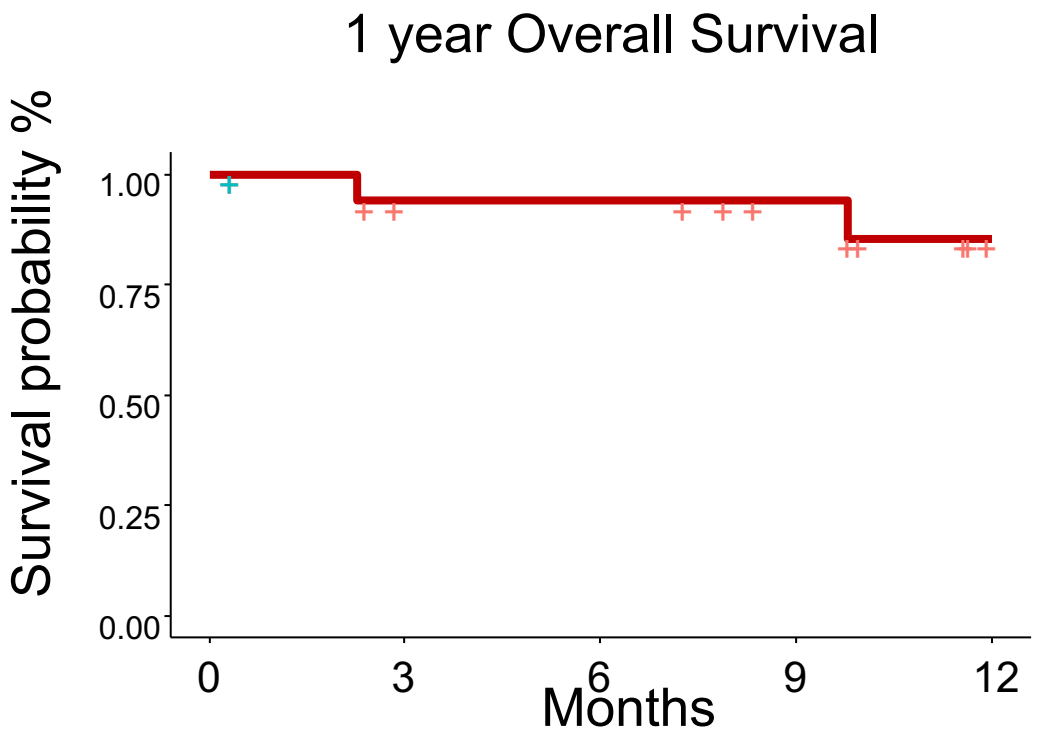
- **FL (n=11): 8 CR, 1PR**
- **DLBCL (n=8): 5 CR**

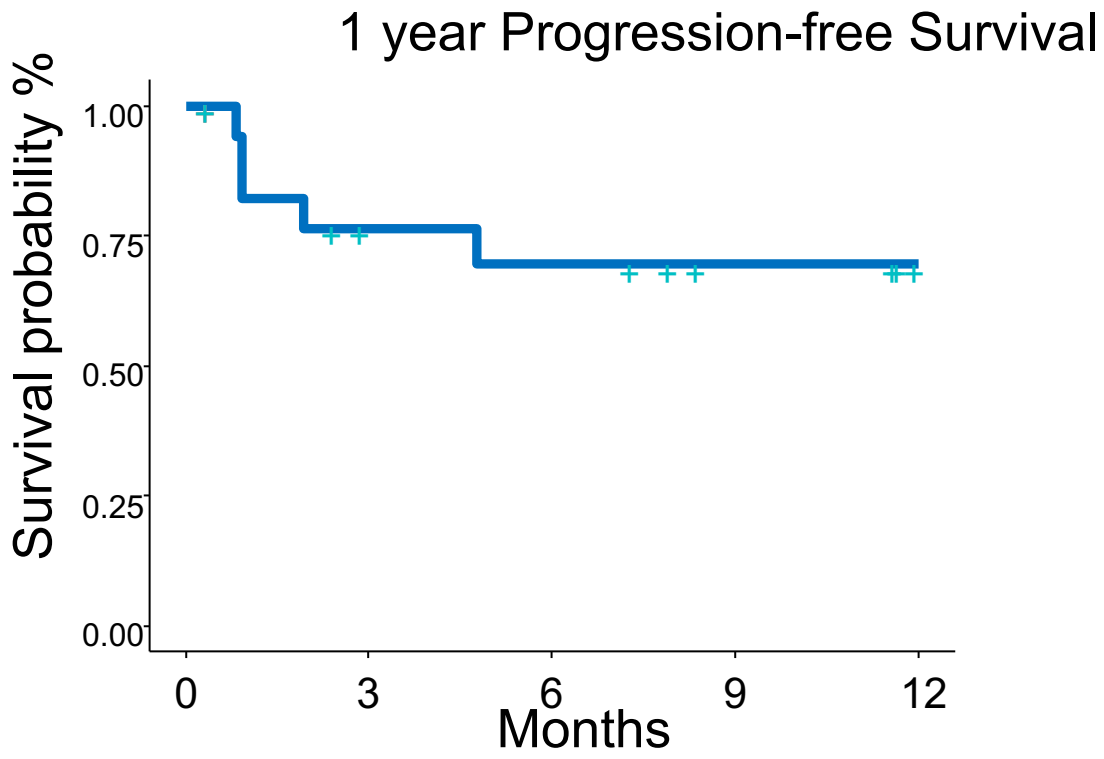
Swimmers' Plot



Bachanova et al., ASH 2020

OS and PFS following GDA201





Median follow-up of alive patients is 10 months (range 1- 28 months)

Phase 1 GDA-201 Study: Conclusions

- GDA-201 is a novel cell product manufactured with nicotinamide without genetic engineering
- GDA-201 target dose of 2 x 10^8 cells/kg in multi-dose infusions is safe and well tolerated
- GDA-201 cells expand in blood, traffic to bone marrow and lymph nodes, and exhibited proliferative phenotype and cytotoxic function.
- Remarkable clinical response of 74% was observed in NHL with almost all complete remissions
- The median duration of response is 10 months with 14 out of 19 patients in ongoing remission
- Future directions include cryopreservation and exploration of multiple treatment cycles.

Data support multi-center Phase 1/2 study in 2021