



# Athenex Cell Therapy:

An Overview of the Transformative Kuur Therapeutics Acquisition

May 2021

NASDAQ:ATNX

[www.athenex.com](http://www.athenex.com)

# Forward Looking Statements

Except for historical information, all of the statements, expectations, and assumptions contained in this press release are forward-looking statements. These forward-looking statements are typically identified by terms such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “foresee,” “goal,” “guidance,” “intend,” “likely,” “may,” “plan,” “potential,” “predict,” “preliminary,” “probable,” “project,” “promising,” “seek,” “should,” “will,” “would,” and similar expressions. Actual results might differ materially from those explicit or implicit in the forward-looking statements. Important factors that could cause actual results to differ materially include: the development stage of our primary clinical candidates and related risks involved in drug development, clinical trials, regulation, uncertainties around regulatory reviews and approvals; our ability to scale our manufacturing and commercial supply operations for current and future approved products, and ability to commercialize our products, once approved; ability to successfully demonstrate the safety and efficacy of its drug candidates and gain approval of its drug candidates on a timely basis, if at all; the preclinical and clinical results for Athenex’s drug candidates, which may not support further development of such drug candidates; risks related to counterparty performance, including our reliance on third parties for success in certain areas of Athenex’s business; our history of operating losses and our need and ability to raise additional capital; uncertainties around our ability to meet funding conditions under our financing agreements and access to capital thereunder; risks and uncertainties inherent in litigation, including purported stockholder class actions; risks and uncertainties related to the COVID-19 pandemic and its potential impact on our operations, supply chain, cash flow and financial condition; competition; intellectual property risks; uncertainties around our ability to successfully integrate acquired and merged businesses in a timely and cost-effective manner and to achieve synergies; risks relating to doing business internationally and in China; the risk of development, operational delays, production slowdowns or stoppages or other interruptions at our manufacturing facilities; and the other risk factors set forth from time to time in our SEC filings, copies of which are available for free in the Investor Relations section of our website at <http://ir.athenex.com/phoenix.zhtml?c=254495&p=irol-sec> or upon request from our Investor Relations Department. All information provided in this release is as of the date hereof and we assume no obligation and do not intend to update these forward-looking statements, except as required by law. Information about the Company and any forward-looking statements contained in this presentation are provided and made only as of 6 May 2021 and should not be relied upon as predictions of future events. The Company assumes no obligation and does not undertake to revise or update forward-looking statements to reflect future events or circumstances, except as required by law.

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# Kuur Therapeutics Acquisition

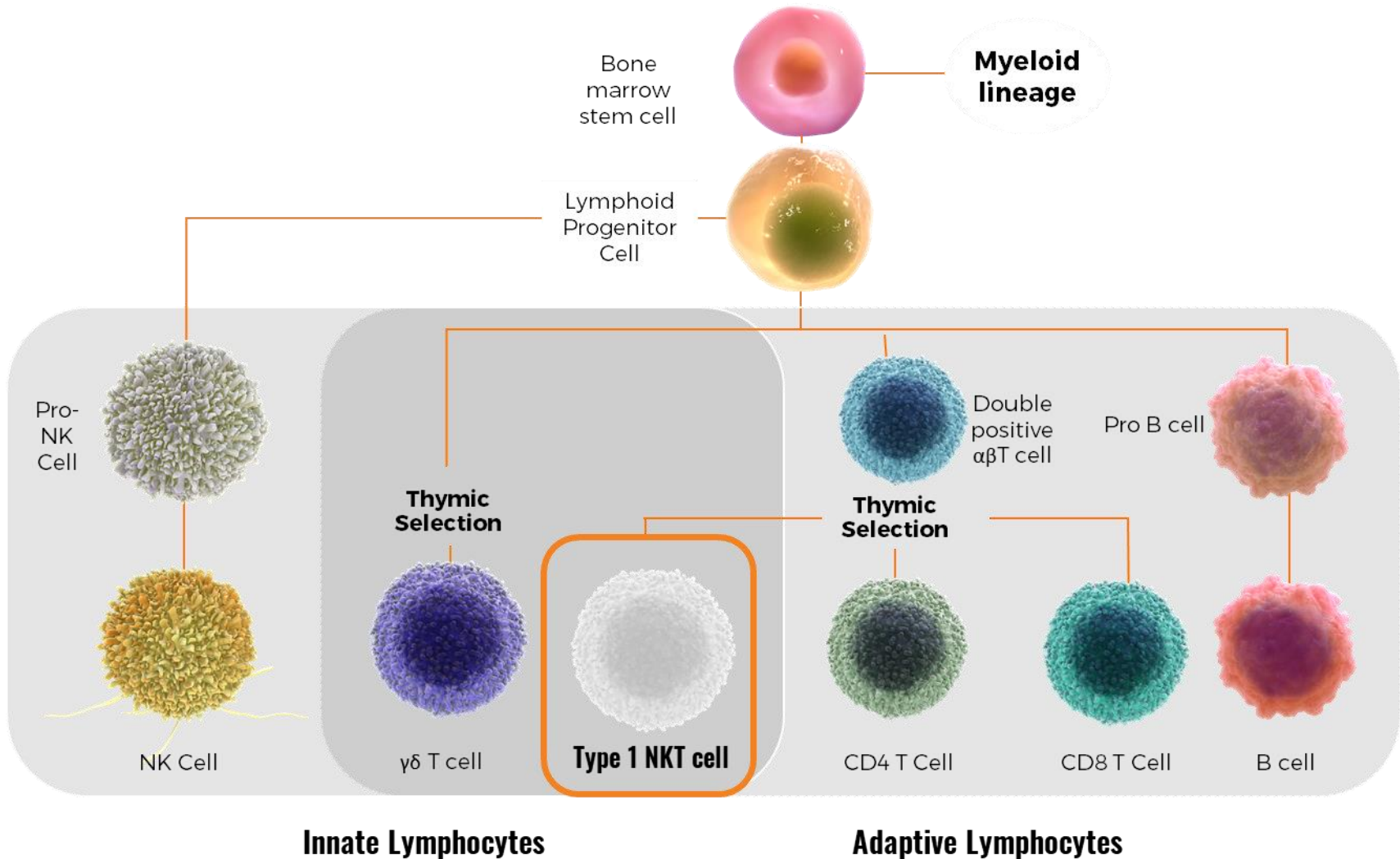
## Attractive Acquisition of a Transformative Cell Therapy Platform

### Kuur Therapeutics Deal Summary

- Innovative company with leading NKT cell technology in allogeneic cell therapy
- Potential first-in-class NKT cell therapy treatment for neuroblastoma and allogeneic platform for hematologic and solid malignancies
- Near-term provides Athenex two attractive assets with solid clinical data and additional data read outs over the next 12 to 18 months
- Long-term provides the opportunity to combine allogeneic NKT cells with TCRs in solid tumors
- Strong IP portfolio on NKT platform technology
- Existing GMP cell manufacturing and viral production through Baylor partnership
- Total consideration of up to \$185 million. Initial \$70 million upfront in primarily Athenex stock, and up to \$115 million in potential milestones payable in Athenex stock or cash (Athenex discretion)

# Kuur Therapeutics Acquisition

NKT Cells Combine the Best Features of NK and T Cells



# Kuur Therapeutics Acquisition




## NKT Cells Compare Favorably to Other Cell Types

	NKT	$\alpha\beta$ T Cells	NK Cells	$\gamma\delta$ Cells	iPSC NK
Bridge the innate immunity and adaptive immunity	✓	-	-	-	-
No need to gene edit out TCR	✓	-	✓	✓	✓
Low GvHD	✓	-	✓	✓	✓
Tumor-Associated Macrophage / Myeloid Derived Suppressor Cell Killing	✓	-	-	-	-
Primary Location and Homing	Tissues	Blood / Tissues	Blood / Tissues	Tissues	Blood / Tissues
Proliferation Post Activation	✓	✓	-	✓	-
Memory / Persistence	✓	✓	-	-	✓

# Kuur Therapeutics Acquisition

## Pipeline Designed to Build a Disruptive Allogeneic Platform

- Autologous proof of concept for CAR-NKT cells for GD2 neuroblastoma
- Allogeneic CAR-NKT cell therapy for hematological cancers

Therapy Type	Product	Indication	Target	Preclinical	Phase 1	Upcoming Milestone
Autologous	<b>KUR-501</b>	Neuroblastoma	GD2			Complete 4 <sup>th</sup> dose level: 1H 2021
Allogeneic	<b>KUR-502</b>	Hematological malignancies	CD19			Complete dose level 1 non-ALL cohort by 1H 2021 and dose level 1 ALL cohort by 4Q 2021
	<b>KUR-503</b>	Hepatocellular carcinoma	GPC3			Submit IND: 1H 2022

# Kuur Therapeutics Acquisition

## Neuroblastoma CAR-NKT Study Targeting GD2 Trial Design

- Development Program: KUR-501 (autologous CAR-NKT)



- **Patients:**
  - Relapsed/refractory high-risk neuroblastoma
  - Outpatient study
- **Manufacturing:**
  - Standard autologous non-mobilized leukopheresis
- **Treatment:**
  - Pretreatment includes cyclophosphamide and fludarabine
  - Infusion: autologous NKT cells engineered to express a GD2-CAR and human IL-15
  - 3+3 Dose escalation:  $3 \times 10^6$ ,  $1 \times 10^7$ ,  $3 \times 10^7$  and  $1 \times 10^8$  CAR-NKT cells/m<sup>2</sup>
- **Follow-Up:**
  - Standard safety, PK (PCR and flow cytometry), immune monitoring and other correlative studies
  - Tumor biopsy at 2 weeks, Marrow aspirate/biopsy if marrow NB involvement at enrollment
  - Imaging: CT/MRI/PET and MIBG using international NB Response Criteria at 4 weeks, additional imaging as clinically indicated

# Kuur Therapeutics Acquisition

## Proof of Concept with CAR-NKT in Neuroblastoma with Efficacy Signs and Good Tolerability

- CAR-NKT cell expansion in peripheral blood in all patients
- CAR-NKT cells homing to tumor and bone marrow by biopsy
- Well-tolerated with excellent safety profile:
  - 1 grade 2 CRS
  - No ICANS
  - Grades 3-4 cytopenias secondary to pre-conditioning
  - Safety profile superior to CAR-T cells
- Protocol amended to add two additional dose levels and option for retreatment
  - Patients 2, 10 and 11 received 2 infusions at indicated dose level
- Patient 11:
  - Bone marrow tumor clearance based on cytology, which is not reflected in Curie score

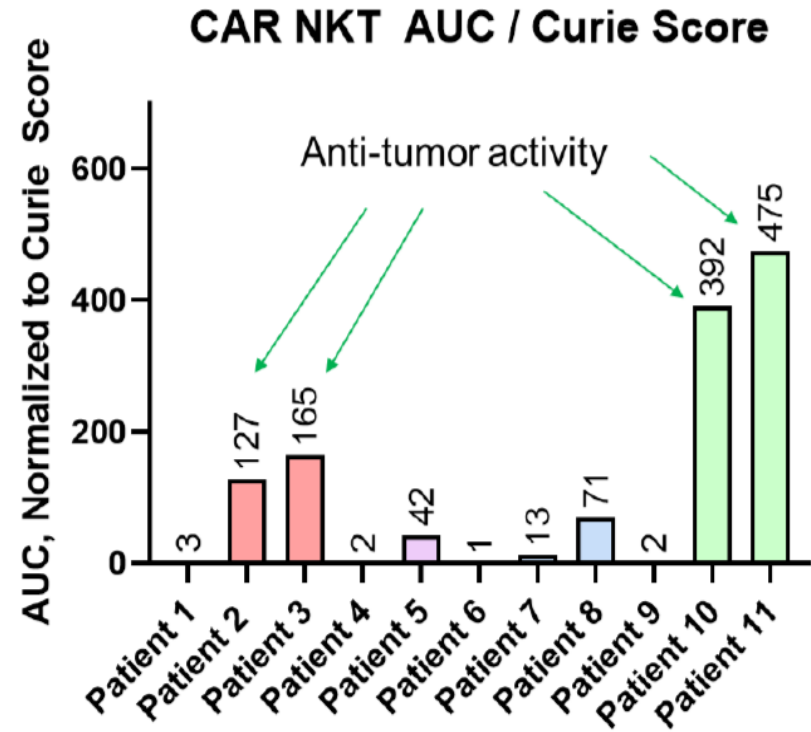
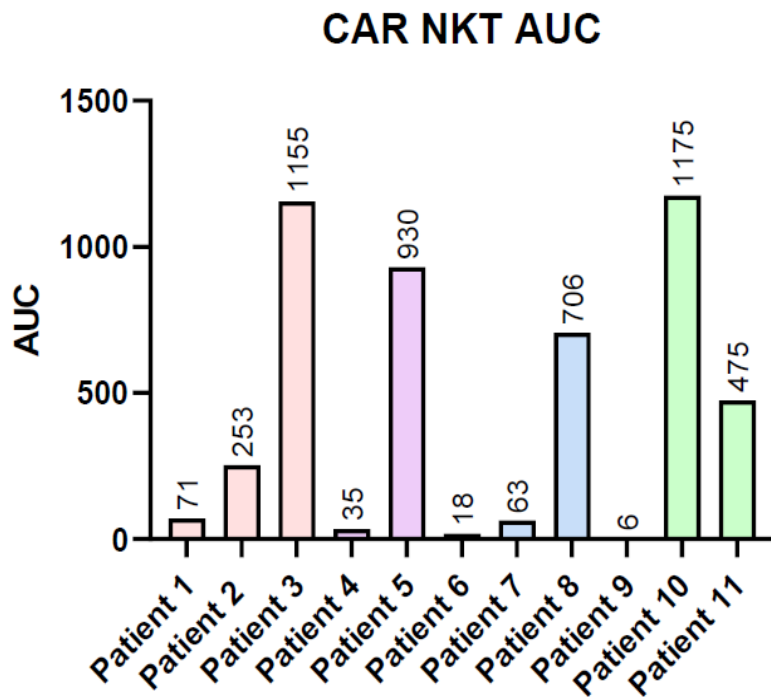
	Patient	Dose	Response	Curie Score		
				Pre	Post	Post 2 <sup>nd</sup> Infusion
Dose Level 1	1	3x10 <sup>6</sup> /m <sup>2</sup>	SD	21	21	
	2	3x10 <sup>6</sup> /m <sup>2</sup>	PR	2	1	SD
	3	3x10 <sup>6</sup> /m <sup>2</sup>	SD	7	5	
Dose Level 2	4	1x10 <sup>7</sup> /m <sup>2</sup>	PD	15	17	
	5	1x10 <sup>7</sup> /m <sup>2</sup>	PD	22	25	
	6	1x10 <sup>7</sup> /m <sup>2</sup>	PD	19	NM	
Dose Level 3	7	3x10 <sup>7</sup> /m <sup>2</sup>	SD	5	5	
	8	3x10 <sup>7</sup> /m <sup>2</sup>	PD	10	21	
	9	3x10 <sup>7</sup> /m <sup>2</sup>	PD	3	NM	
Dose Level 4	10	1x10 <sup>8</sup> /m <sup>2</sup>	PR	3	1	CR
	11	1x10 <sup>8</sup> /m <sup>2</sup>	SD	1	1	PD



# Kuur Therapeutics Acquisition

Updated GINAKIT2 Data Presented at ASGCT

**Tumor Burden and CAR-NKT Expansion Are Associated with Antitumor Activity in Children with Neuroblastoma**



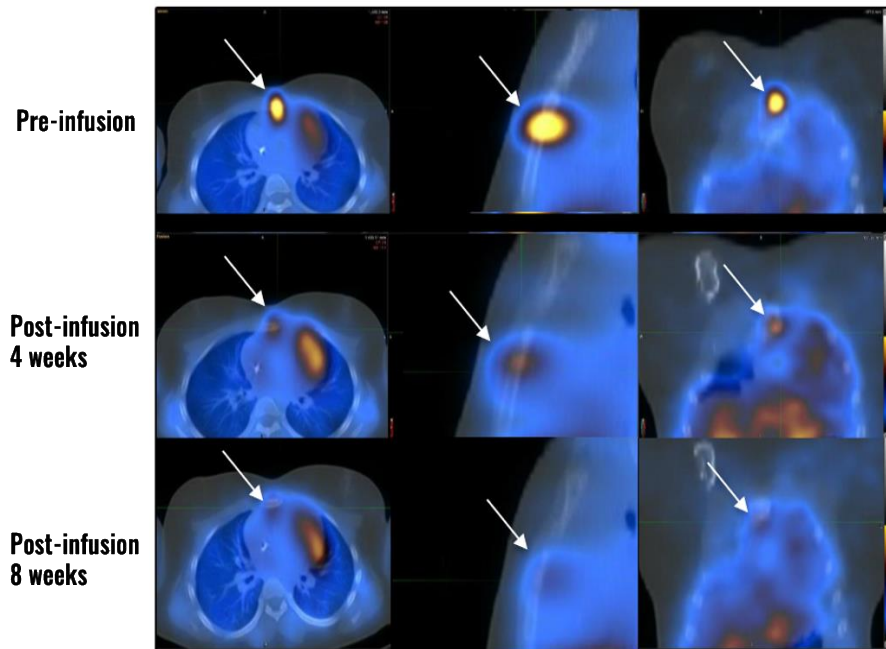
# Kuur Therapeutics Acquisition

## GD2 CAR-NKT Efficacy

One Complete Response, One Partial Response and Four Patients with Stable Disease Out of 11 Patients Treated

Duration of response approximately 3 months and ongoing when patient withdrew from study.

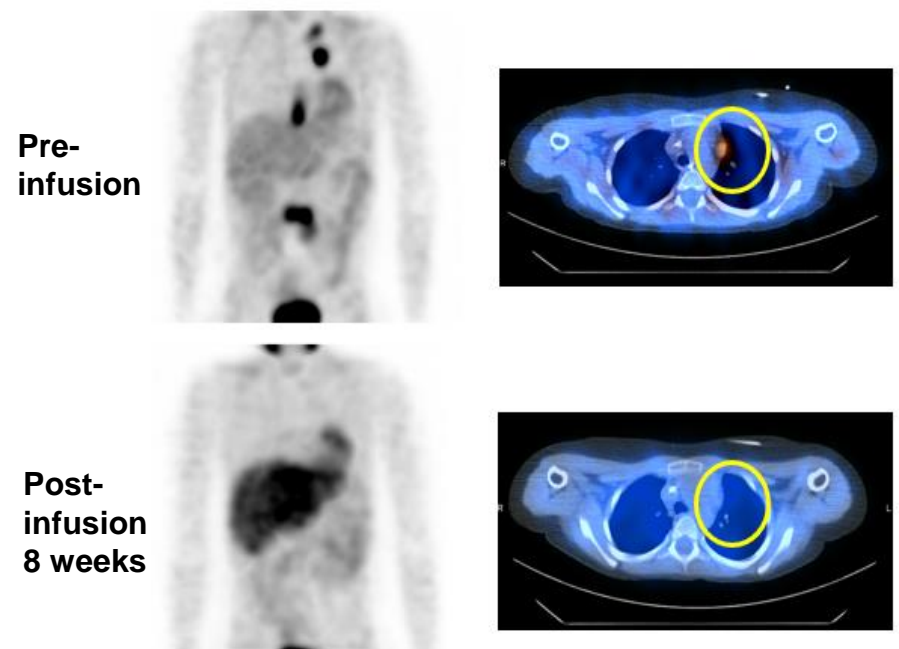
**PR: Patient 2**



Heczey et al, *Nat Med* (2020).

Duration of response approximately 5 months and currently ongoing. Post-infusion uptake in heart, liver and bladder is physiological, not related to neuroblastoma.

**CR: Patient 10**



# Kuur Therapeutics Acquisition

## Allogeneic CAR-NKT Targeting CD19 Trial Design

- Development Program: KUR-502 (allogeneic CAR-NKT)

### ANCHOR



- **Patients:**

- Adults with relapsed/refractory CD19+ leukemia and lymphoma (includes: ALL, DLBCL, TFL, CLL)
- Previous autologous CAR-T therapy allowed
- Outpatient study

- **Manufacturing:**

- Unmatched healthy donor, standard non-mobilized leukapheresis

- **Treatment:**

- Pre-treatment includes cyclophosphamide and fludarabine
- Infusion: allogeneic NKT cells engineered to express a CD19-CAR, human IL-15, and shRNAs to down-regulate HLA
- Bayesian optimal interval (BOIN) dose escalation:  $1 \times 10^7$ ,  $3 \times 10^7$ , and  $1 \times 10^8$  CAR-NKT cells/m<sup>2</sup>
- Patients enrolled in 2 cohorts: ALL and non-ALL

- **Follow-Up:**

- Standard safety, PK (PCR and flow cytometry), immune monitoring and other correlative studies
- Accessible lymph node biopsy at 3-6 weeks, if marrow involvement, aspirate and biopsy will be collected pre-infusion at two- and four-weeks post-infusion
- Imaging: CT/MRI/PET and MIBG using international NB Response Criteria at 4 weeks, additional imaging as clinically indicated

# Kuur Therapeutics Acquisition

## Encouraging Efficacy and Safety Data with Several Additional Correlative Studies in Progress

### Safety

- No CRS, ICANS, or GvHD
- Grades 3-4 cytopenias secondary to pre-conditioning

Patient	Dose	Best Response
1	1x10 <sup>7</sup> /m <sup>2</sup>	PR
2	1x10 <sup>7</sup> /m <sup>2</sup>	CR

### Additional Correlative Studies

- Homing and persistence of allogeneic CAR-NKT cells in lymph node 5 weeks after infusion documented in one patient
- Persistence and expansion of CAR-NKT in peripheral blood
  - Transient persistence observed at lowest dose level
- Epitope spreading for evidence of introduction of patient anti-tumor immunity
- Single cell sequencing of cDNA from patient biopsies
- Flow cytometry of patient lymphoid and myeloid subsets
- Post treatment patient plasma cytokine levels

### Absolute B Cells

