

Autolus

# Developing Next Generation Programmed T Cell Therapies

January 2025



Autolus.com

# Disclaimer

These slides contain forward-looking statements within the meaning of the “safe harbor” provisions of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical facts, and in some cases can be identified by terms such as “may,” “will,” “could,” “expects,” “plans,” “anticipates,” and “believes.” These statements include, but are not limited to, statements regarding Autolus’ development of its product candidates, including the obe-cel program; the profile and potential application of obe-cel in additional disease settings; the future clinical development, efficacy, safety and therapeutic potential of the Company’s product candidates, including progress, expectations as to the reporting of data, conduct and timing and potential future clinical and preclinical activity and milestones; expectations regarding the initiation, design and reporting of data from clinical trials and preclinical studies; the extension of the pipeline beyond obe-cel; expectations regarding the regulatory approval process for any product candidates; the benefits of the collaboration between Autolus and BioNTech, including the potential and timing of milestone payments and royalties under the terms of the strategic collaboration; the Company’s current and future manufacturing capabilities; and the Company’s anticipated cash runway. Any forward-looking statements are based on management’s current views and assumptions and involve risks and uncertainties that could cause actual results, performance, or events to differ materially from those expressed or implied in such statements. These risks and uncertainties include, but are not limited to, the risks that Autolus’ preclinical or clinical programs do not advance or result in approved products on a timely or cost effective basis or at all; the results of early clinical trials are not always being predictive of future results; the cost, timing and results of clinical trials; that many product candidates do not become approved drugs on a timely or cost effective basis or at all; the ability to enroll patients in clinical trials; and possible safety and efficacy concerns. For a discussion of other risks and uncertainties, and other important factors, any of which could cause Autolus’ actual results to differ from those contained in the forward-looking statements, see the section titled “Risk Factors” in Autolus’ Annual Report on Form 10-Q filed with the Securities and Exchange Commission, or the SEC, on November 12, 2024, as well as discussions of potential risks, uncertainties, and other important factors in Autolus’ subsequent filings with the Securities and Exchange Commission. All information in this presentation is as of the date of the presentation, and Autolus undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise, except as required by law. You should, therefore, not rely on these forward-looking statements as representing the Company’s views as of any date subsequent to the date of this presentation.

# Autolus is positioned for commercial execution and market expansion

Obe-cel product franchise supports multiple growth opportunities

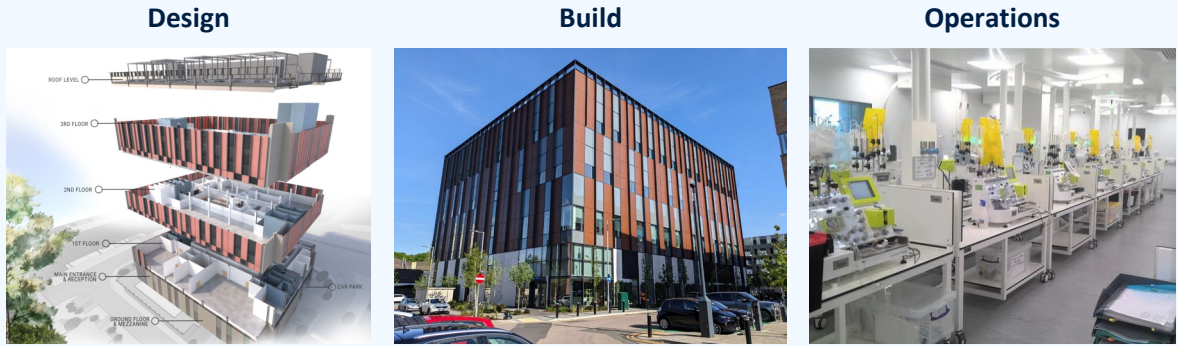


- EU/UK approvals expected 2H 2025
- Expanding obe-cel opportunity in hem-oncology and autoimmune diseases
- Developing early-stage pipeline of novel CAR-T therapies

Obe-cel product family	Product	Indication	Target	Preclinical	Phase 1	Phase 2/ Pivotal	Approved
	AUCATZYL®	Adult ALL	CD19	[Progress bar]			
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## Commercial execution and market expansion supported by:

### In-house, purpose-built manufacturing facility



### Strategic collaborations and strong cash position

**\$657M as of Q3 2024**





**AUTOLUS' FIRST APPROVED PRODUCT**

**AUCATZYL®**

A potentially best-in-class, standalone  
CD19 CAR T cell therapy

# AUCATZYL® now FDA approved



Please see full prescribing information [Prescribing information](#)

- ✓ **AUCATZYL indicated for the treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (B-ALL)**
- ✓ **First chimeric antigen receptor T-cell (CAR T) therapy approved by the FDA with no requirement for a REMS program (Risk Evaluation Mitigation Strategy)**
- ✓ **Novel and differentiated mechanism of action: first and currently only approved CD19 CAR T with a fast off-rate**
- ✓ **First and currently only approved CAR T therapy with customized, tumor-burden guided dosing**

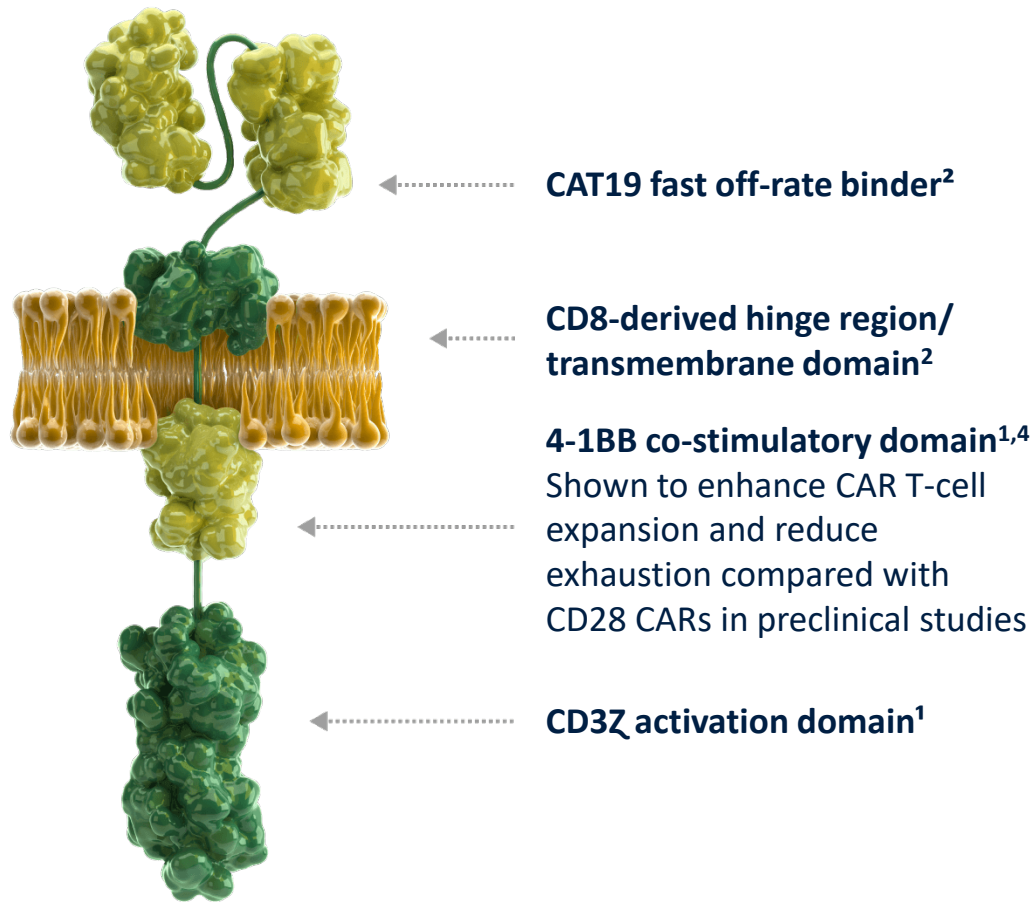
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# Important Safety Information

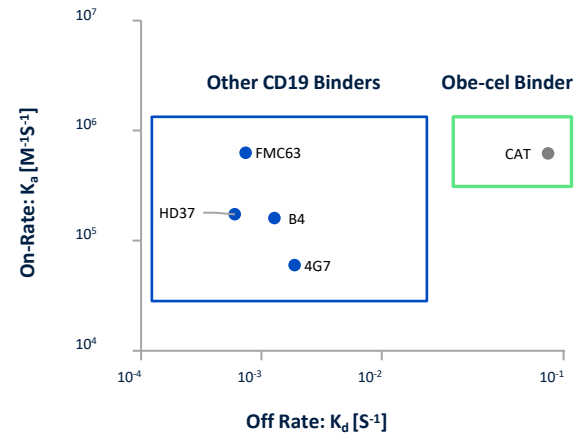
- The safety of AUCATZYL includes a boxed warning for CRS, neurologic toxicities, and secondary hematological malignancies. ICANS, including fatal or life-threatening reactions, occurred in patients receiving AUCATZYL. T-cell malignancies have occurred following treatment of hematologic malignancies with BCMA- and CD19-directed genetically modified autologous T-cell immunotherapies.
- In the FELIX trial, severe, including life-threatening and fatal infections occurred in patients after AUCATZYL infusion. The non-COVID-19 infections of all grades occurred in 67% (67/100) of patients. Grade 3 or higher non-COVID-19 infections occurred in 41% (41/100) of patients.
- Please see full [Prescribing Information](#), including **BOXED WARNING** and Medication Guide.

# We believe AUCATZYL<sup>®</sup> has a unique mechanism of action

Clinical data show increased activity and reduced toxicity



## Fast off-rate



Shorter half-life of interaction compared to binders used in approved products

- obe-cel = 9.8 seconds
- Kymriah<sup>®</sup> = 21 minutes

## Potential for improved potency, reduced toxicity

Avoided over-activation of CAR T cells



Reduced toxicities

Increased CAR T peak expansion



Improved peak activity and persistence

Avoided exhaustion of CAR T-cells

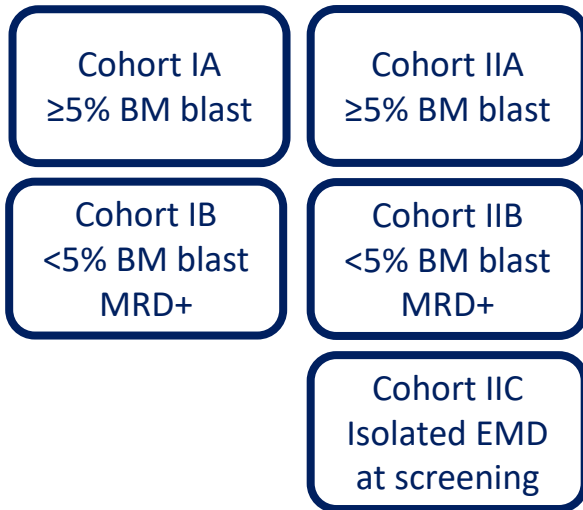


Improved engraftment  
Improved persistence

# AUCATZYL was approved based on results from the FELIX trial



## FELIX Phase 1b/2



Patients (N)	Ph1b/2 pooled <sup>1</sup>
Enrolled	153
Infused	127

## Background

- Open-label, multinational, single-arm Phase 1b/2 trial in adult patients with R/R B-ALL<sup>1-2</sup>; largest CAR T cell therapy trial in R/R B-ALL to date (N=153 enrolled)
- Conducted during COVID-19 pandemic with highly immune compromised patients

## Summary of Trial Experience

- High ORR, encouraging EFS/OS and favorable tolerability with low levels of high-grade CRS and ICANS
- Timely and reliable clinical product supply and logistics despite COVID-19 pandemic restrictions
- Across all Phase 1b/2 cohorts, 40% of responders in ongoing remission without subsequent stem cell transplant/other therapy<sup>1</sup>
- Survival outcomes suggesting potential of long-term plateau<sup>1</sup>

<sup>1,2</sup> Roddie C, et al "Obecabtagene autoleucel in B-cell acute lymphoblastic leukemia" N Engl J Med 2024; DOI: 10.1056/NEJMoa2406526



# FELIX trial published in New England Journal of Medicine<sup>1</sup>

Favourable response rate and tolerability, despite challenging patient population

## High overall response rate with deep molecular responses

- Durable responses, particularly in patients with a low-to-intermediate bone marrow burden

Response by disease status at lymphodepletion	Overall Remission Rate (CR/CRi)
All patients (n=127)	77%
Morphological disease (n=91)	75%
Measurable residual disease (n=29)	96%
Isolated extramedullary disease (n=7)	71%

## Excellent tolerability profile

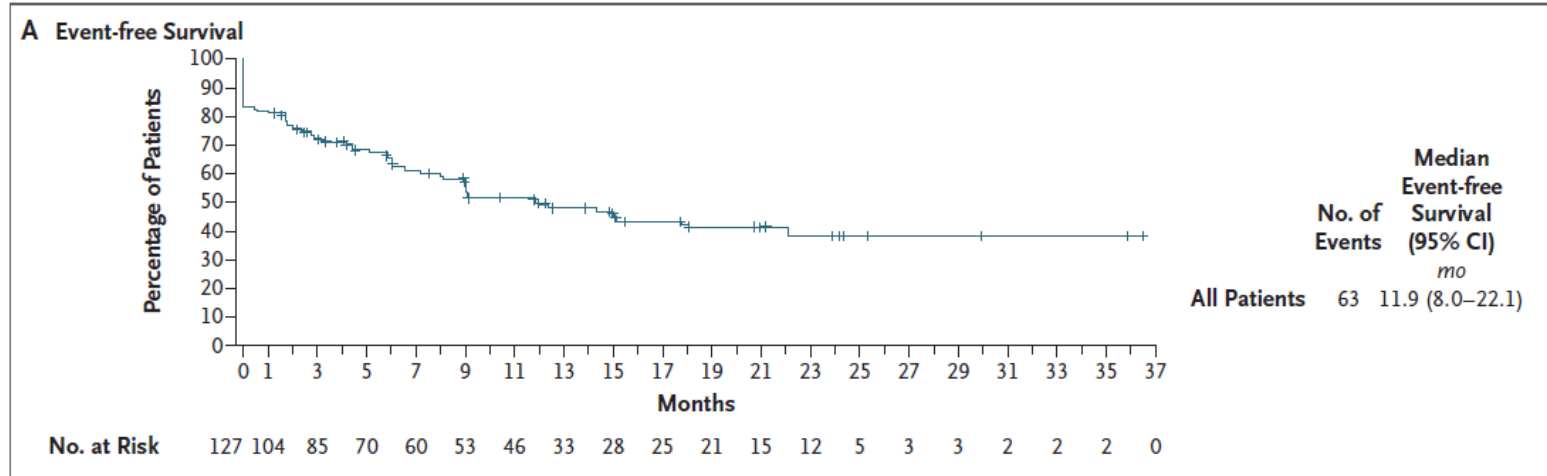
- Very low rates of high-grade immunotoxicities
- No high-grade events in low disease burden patients

Safety by disease burden at lymphodepletion	Grade ≥3 CRS	Grade ≥3 ICANS
All patients (n=127)	2%	7%
>75% Blasts (n=40)	2%	12%
5-75% Blasts (n=51)	4%	8%
<5% Blasts (n=36)	0%	0%

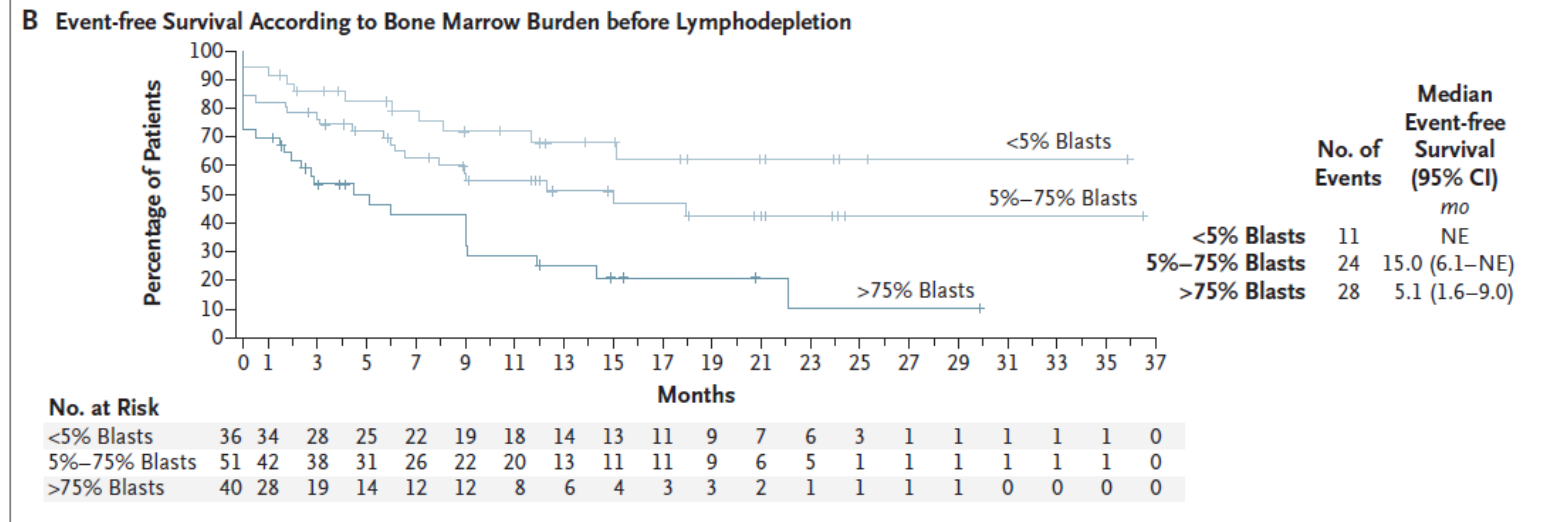
# Deep molecular responses result in long term remissions in adult ALL

Survival outcomes show potential of long-term plateau with 12-month EFS rates 49.5%

- In all patients, the median EFS was 11.9 months



- Lower disease burden at lymphodepletion was associated with better outcomes

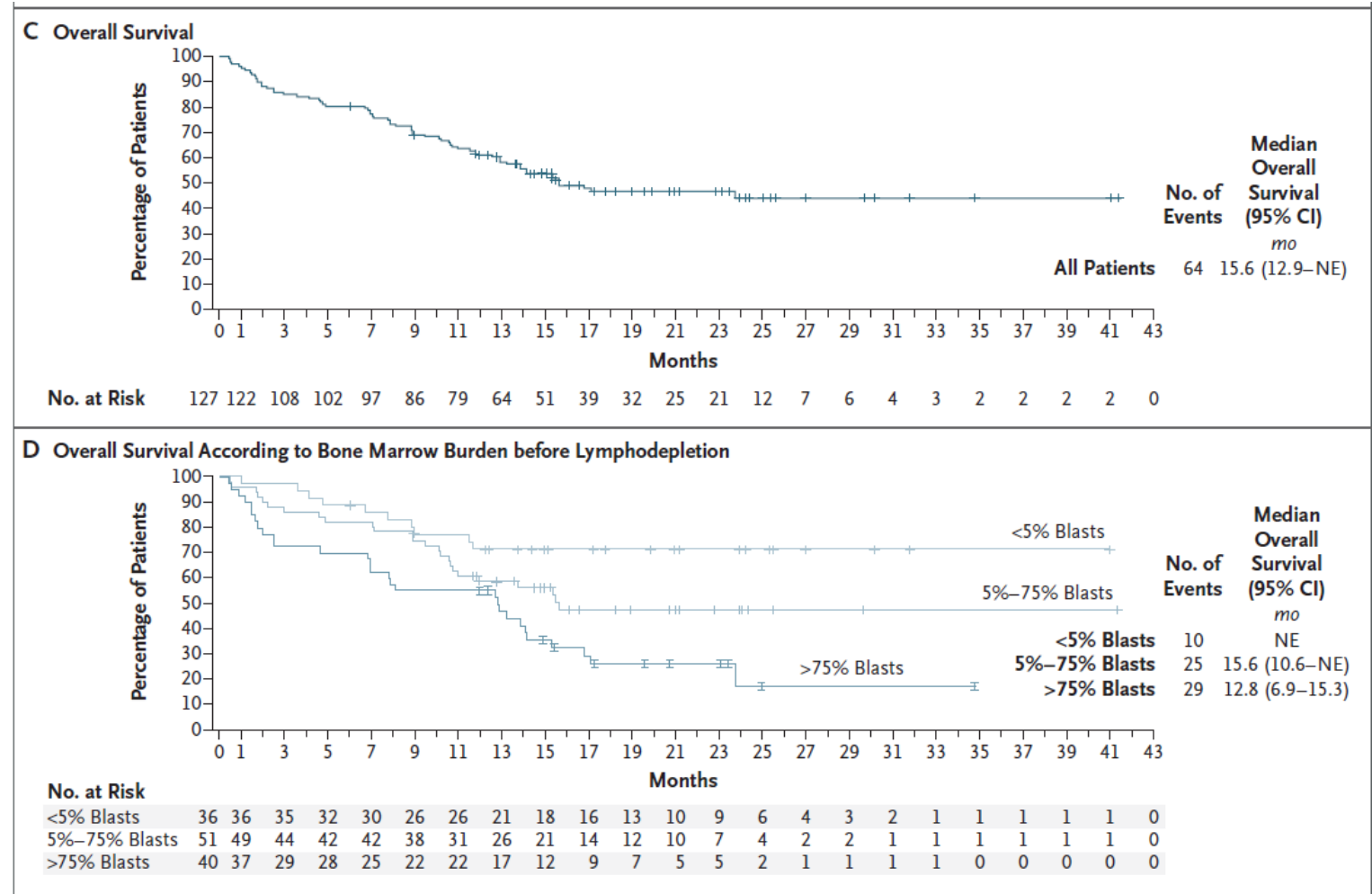


# Deep molecular responses result in long term remissions in adult ALL

Estimated 6- and 12-month overall survival rates were 80.3% and 61.1%, respectively

- In all patients, the median OFS was 15.6 months

- Lower disease burden at lymphodepletion was associated with better outcomes



# AUCATZYL<sup>®</sup> is poised to fill the unmet need for r/r ALL patients

- We believe AUCATZYL is a transformative product in the r/r ALL space
- Unique MOA designed to deliver potency and persistency that results in deep and durable efficacy
- Favorable tolerability profile
- Customized tumor-burden guided dosing
- Well-positioned to deliver therapy globally with Autolus' proven reliable manufacturing





## Commercial Launch

# Pillars to drive commercial success

## Prioritizing authorization of centers Post-approval

**30** key centers primed for activation  
covering ~ 60% of r/r B-ALL target population  
with **~30** additional centers to follow by end 2025

## Team dedicated to successful commercial efforts

**Experienced team** with multiple CAR T launches  
Strong **scientific communication** and **physician  
engagement** within medical affairs  
**Dedicated single point-of-contact** for every center

## Scalable, efficient and reliable supply

**The Nucleus:** Autolus' state-of-the-art, dedicated  
purpose-built facility

Target vein-to-release time  
of **~16 days**



## Pricing strategy focused on delivering value to customers and achieving broad coverage

**\$525,000**  
WAC<sup>1</sup>

Pricing reflects clinical evidence, differentiated safety  
profile, economic value

<sup>1</sup>Wholesale acquisition cost, or WAC, before any discounts, rebates or other price concessions

# AUCATZYL® Authorized Treatment Centers

24 Centers Authorized as of January 10, 2025



**Near-Term Plan:**

**30 centers**  
covering  
**60% of target**  
**population**

**End of 2025:**

**~60 centers**  
covering  
**90% of**  
**population**

# The Nucleus: Manufacturing facility supports commercial execution

State of the art design and in-house operations established – groundbreaking to complete validation in 2 years

- Designed for 2,000+ batches per year
- Timeline to validation reduced by ~60% compared to prior CAR T facilities
- Target vein to delivery 16 days at launch



**Purpose-built facility can be efficiently replicated as supply demands increase**



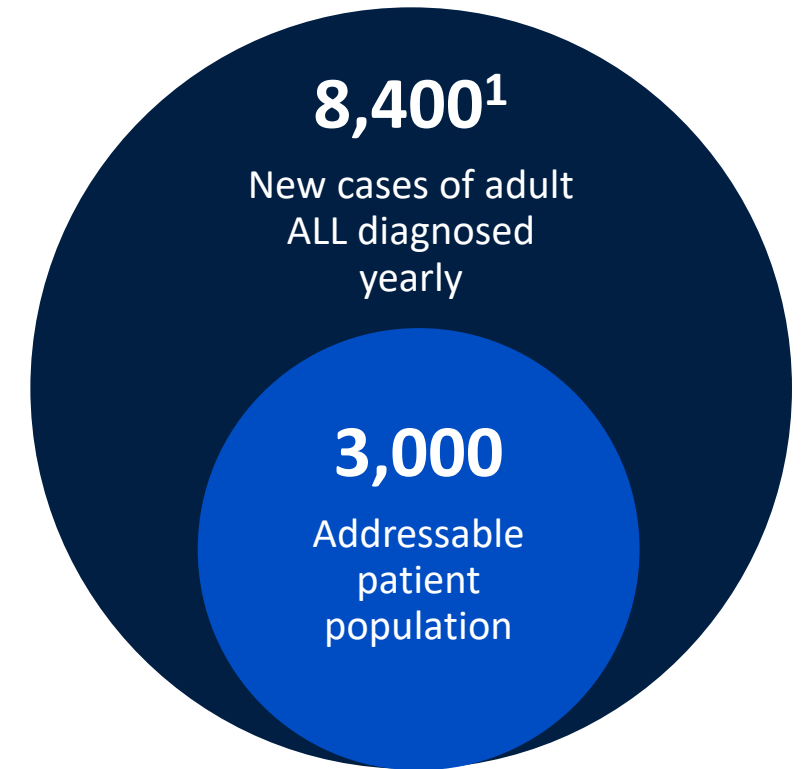


ALL: unmet need  
and market overview

# Over 8,000 new cases of adult ALL annually worldwide

Successful therapy requires high level of activity and sustained persistence paired with good tolerability

- Median overall survival is < 1 year in r/r adult ALL
- 1<sup>st</sup> line therapy is based on high dose chemotherapy cycles given over a period of 12 – 36 months
- In 1<sup>st</sup> line therapy approx. 90% of patients achieve a CR, but most patients relapse
- Blincyto<sup>®</sup> is incorporated into frontline therapy as an additional component
- Aucatzyl<sup>®</sup> offers opportunity as a standalone therapy for patients in 2<sup>nd</sup> and subsequent lines of therapy

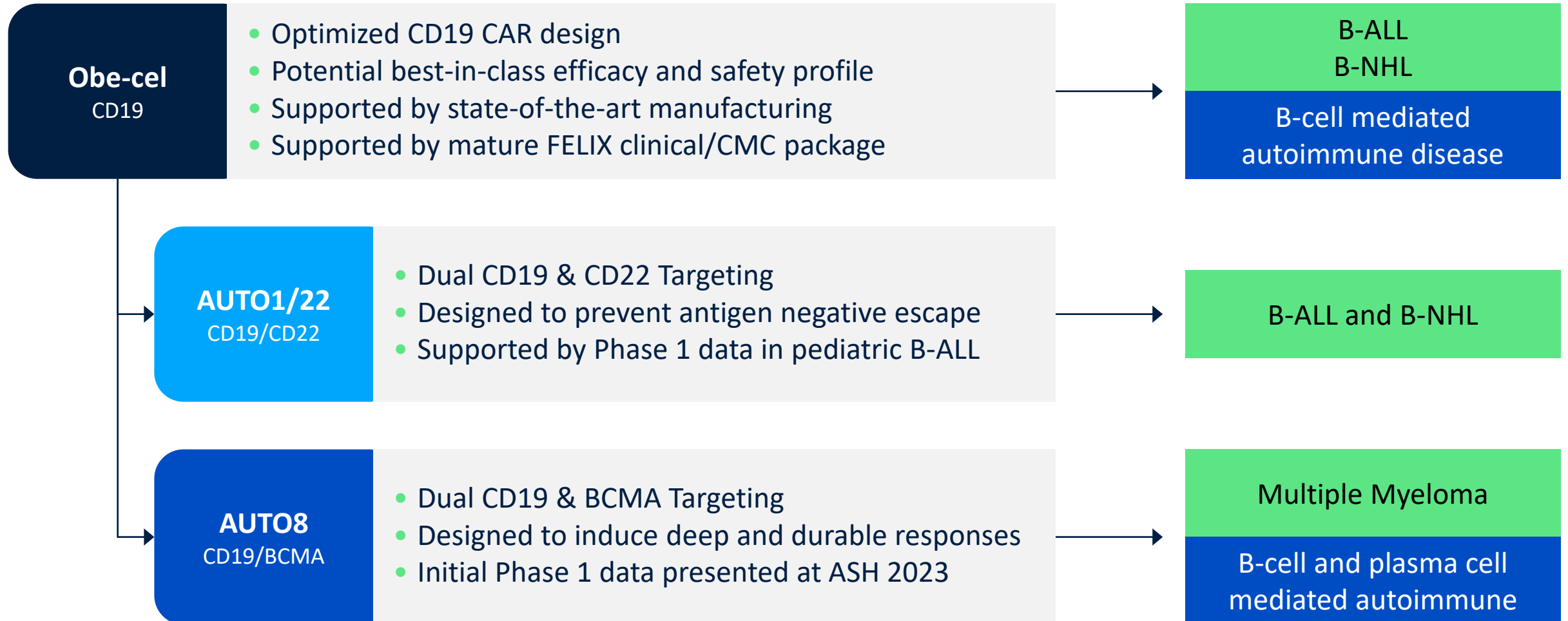


# Expanding the obe-cel opportunity

Deep value program with potentially broad applicability

# The obe-cel product family and franchise opportunity

Potential value-creation through multiple life-cycle management and market expansion opportunities



# MOA and established commercial capabilities are key differentiators

Obe-cel is the only CD19 CAR with an FDA approval outside of autoimmune disease

## Autolus Potential Advantage

- ✓ Favorable tolerability to drive acceptability in non-oncology indications
- ✓ Deep cut into the CD19+ B and plasma cell
- ✓ Robust, economical and scalable manufacturing and established commercial infrastructure
- ✓ Potential for accelerated clinical program
- ✓ Only FDA-approved CAR-T therapy in development for autoimmune indications

**Supports differentiated approach and potential for obe-cel in autoimmune disease areas**

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# Phase 1 study in r/r SLE – enrollment ongoing

Primary goal of the Phase 1 study will be confirming the fixed dose in adult SLE patients

## CARLYSLE Study

A Single-Arm, Open-Label, Phase I Study to Determine the Safety, Tolerability and Preliminary Efficacy of Obecabtagene Autoleucel in Patients with Severe, Refractory Systemic Lupus Erythematosus (SLE)\*

### Study design summary

- n: 6 (option to add cohort of 6 patients)
- Primary endpoint: establish tolerability and safety of obe-cel in patients with severe, refractory SLE
- Secondary endpoints: evaluate preliminary efficacy of obe-cel using measures of SLE disease activity
- Dosing:  $50 \times 10^6$  CD19 CAR-positive T cells
- Follow up: up to 12 months
- 3 centers enrolling in UK and Spain

### Status and updates

- Initial cohort (n=6); expect completion of patient dosing in Q1 2025
- Initial patient data in Q1 2025
- Presentation of full data with follow-up targeted for 2H 2025 at a medical conference

# Partnerships, pipeline programs and technologies

A broad portfolio of potential next  
generation modular T cell therapies

# Pipeline supports growth with multiple development opportunities

## Obe-cel product family

Product	Indication	Target	Preclinical	Phase 1	Phase 2/Pivotal	Approved	Status
AUCATZYL®	Adult ALL	CD19					MAA review EMA & MHRA
obe-cel	Systemic Lupus Erythematosus	CD19					Initial data Q1 2025
obe-cel	Pediatric ALL	CD19					Initial data H2 2025
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## Additional programs

Product	Indication	Target	Preclinical	Phase 1	Phase 2/Pivotal	Approved	Status
AUTO4	TRBC1+ Peripheral TCL	TRBC1					Data in peer reviewed journal
AUTO5	TRBC2+ Peripheral TCL	TRBC2					Data in peer reviewed journal
AUTO6NG*§	Neuroblastoma	GD2					Open and recruiting
AUTO9*	Acute Myeloid Leukemia	CD33,123,CLL1					Initiate Ph1 2025

\*UCL Collaboration

§ BioNTech holds option to co-fund and co-commercialise



# Leveraging our industry leading technology platform via partnerships

## Technology partnerships

Leveraging our modular programming technology to generate safer and more effective therapies

Tumor targeting, pharmacological control and activity enhancement for cellular therapies

Validating collaborations with leading pharma and biotech companies

Potential for value creation through near term option exercise fees, milestone payments and royalties from net sales

**BIONTECH**

Leveraging technology platform for BioNTech's programs

 Bristol Myers Squibb

Access to the RQR8 safety switch for selected cell therapy programs for the treatment of cancer

**moderna**

Access to proprietary binders for the development of mRNA-based therapeutics for the treatment of cancer



Upcoming news flow

# Autolus planned news flow

Anticipated Milestone or Data Catalysts	Anticipated Timing
Obe-cel FELIX data update at ASH 2024	December 2024
Initial data from SLE Phase 1 trial	Q1 2025
Obe-cel UK and EU approvals	2H 2025
Initial data from PY01 trial in pediatric ALL	2H 2025
SLE Phase 1 trial presentation at medical conference	2H 2025

Oncology **Autoimmune**

# Summary

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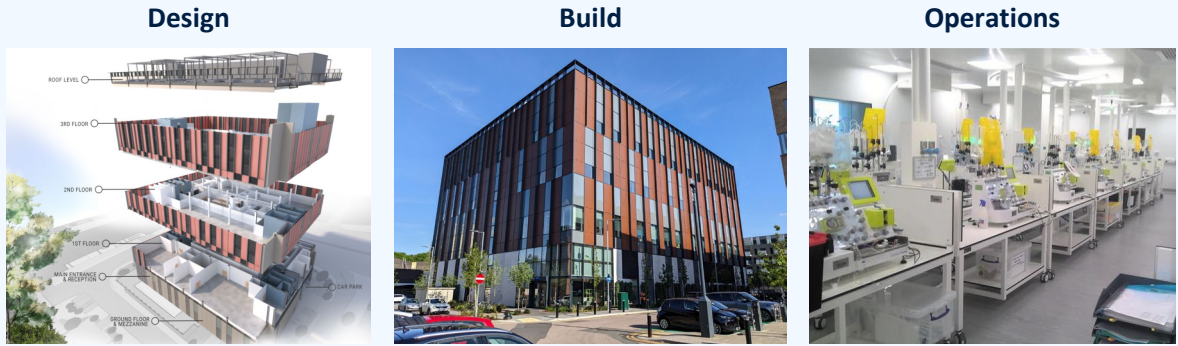


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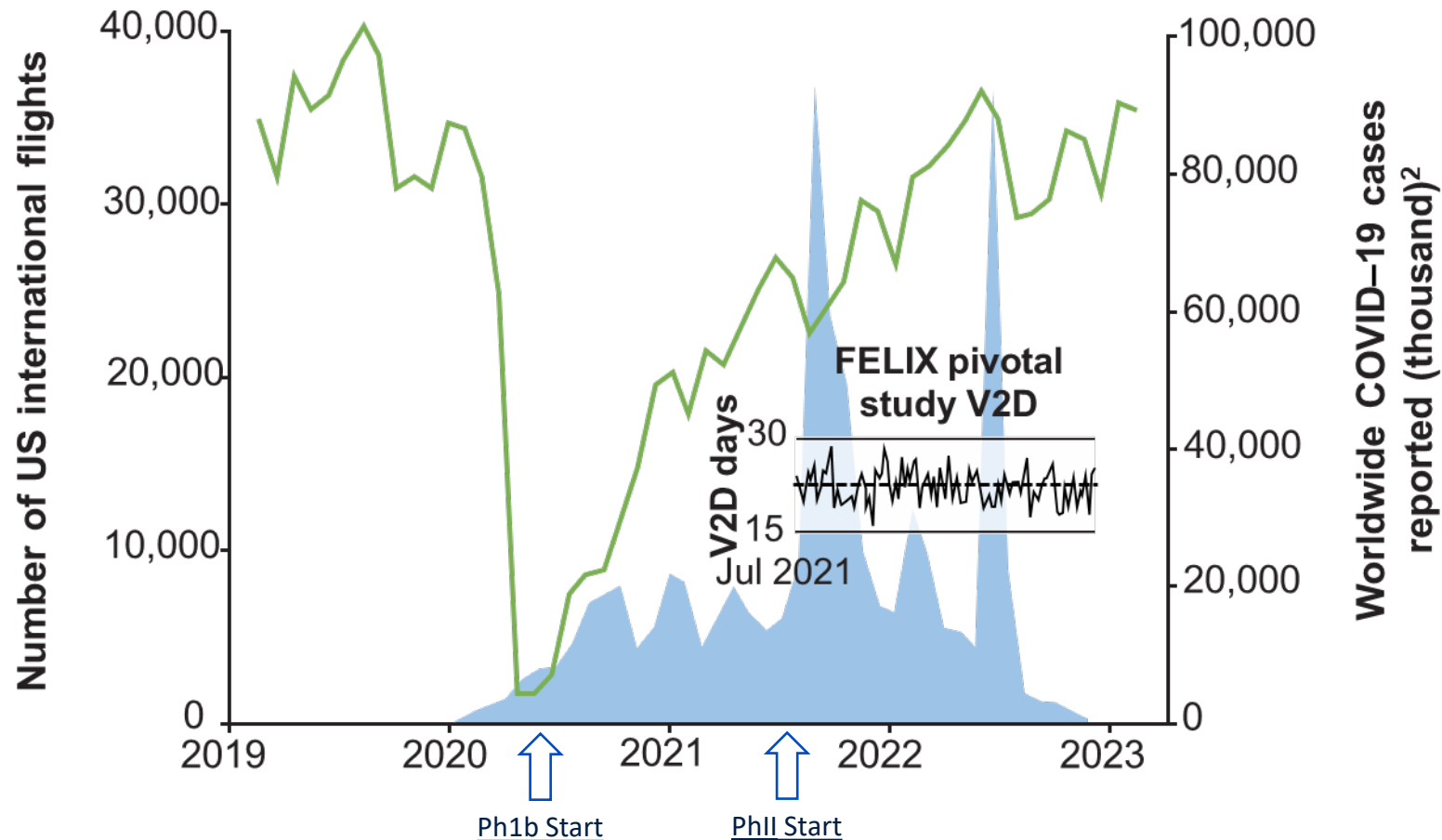
Thank you

Autolus

# Appendix

# The FELIX phase 1b/2 pivotal study

## Reliable obe-cel supply for FELIX despite the COVID-19 pandemic



- US international airline flights decreased by 41% compared to flights from pre-COVID-19 pandemic<sup>1</sup>
- BUT international flights are reliable and on time
- Sample collection and drug product delivery were successfully maintained, with no batches impacted

<sup>1</sup>United States Department of Transportation, Bureau of Transportation Statistics 2021 [online]. Available at: <https://www.bts.gov/data-spotlight/commercial-aviation-2020-downturn-airline-passengers-employment-profits-and-flights> Accessed October 2023;

<sup>2</sup>World Health Organization COVID-19 dashboard [online]. Available at: <https://covid19.who.int/> Accessed October 2023



# Strategic multi-platform R&D collaboration with BioNTech

## CAR T Cell Therapies

BioNTech to financially support obe-cel planned/potential commercial launch in adult ALL (Acute Lymphoblastic Leukemia) and expansion of development program

## Development Product Options

BioNTech to receive co-development and co-commercialization options for AUTO1/22 (CD19/22) and AUTO6NG (GD2) programs

## Commercial Infrastructure Access

BioNTech to receive option to access Autolus' GMP product supply and commercial infrastructure for their CAR T program, BNT211

## Technology Platform License

BioNTech to receive license and options to access proprietary binders, safety switches and technologies for certain BioNTech programs

## Deal Financials

### Upfront Payments

- \$200 million upfront for equity
- \$50 million upfront cash

### Downstream Economics

- Up to \$580 million in further option exercise and milestones payments
- BioNTech to receive up to mid-single digit royalty on obe-cel project financing
- Autolus eligible for an additional equity investment of \$20m, an option exercise payment and profit share based on products manufactured for BioNTech's BNT211 program
- BioNTech has option to co-fund and co-commercialize AUTO1/22 and AUTO6NG, if approved, in return for profit share
- Technology license and options provided in exchange for milestones and royalties