

# Precigen, Inc.

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## IMMUNO-ONCOLOGY

PRODUCT	PLATFORM	INDICATION	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
PRGN-3005	UltraCAR-T	Ovarian Cancer					
PRGN-3006	UltraCAR-T	AML, MDS					
PRGN-3007	UltraCAR-T	ROR1+ Hematological & Solid Tumors					
PRGN-2009	AdenoVerse	HPV+ Solid Tumors					

## INFECTIOUS DISEASE

PRODUCT	PLATFORM	INDICATION	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
PRGN-2012	AdenoVerse	Recurrent Respiratory Papillomatosis (RRP)					

## AUTOIMMUNE DISORDERS

PRODUCT	PLATFORM	INDICATION	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
AG019	ActoBiotics	Type 1 Diabetes					

## EMERGING THERAPEUTICS

PRODUCT	PLATFORM	INDICATION	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
INXN-4001	Non-viral UltraVector	Heart Failure					



**PRGN-3006 UltraCAR-T: Completed dose escalation and presented positive interim data from the Phase 1 trial in AML**



**PRGN-3005 UltraCAR-T: Initiated the IV arm of the Phase 1 trial and completed enrollment for Dose Level 3. Presented encouraging interim data from Phase 1 IP arm**



**PRGN-3007 next gen UltraCAR-T incorporating intrinsic PD-1 inhibition: Received IND clearance to initiate a Phase 1 trial in ROR1<sup>+</sup> hematological (CLL, MCL, ALL, DLBCL) and solid (TNBC) tumors**



**PRGN-2012 AdenoVerse Immunotherapy: Completed enrollment in the dose escalation and dose expansion cohorts of the Phase 1 trial in Recurrent Respiratory Papillomatosis and presented positive preliminary data**



**PRGN-2009 AdenoVerse Immunotherapy: Initiated Phase 1 checkpoint combination and Phase 2 monotherapy trials. Presented positive data from the Phase 1 monotherapy and checkpoint combination trial in HPV-associated cancers**



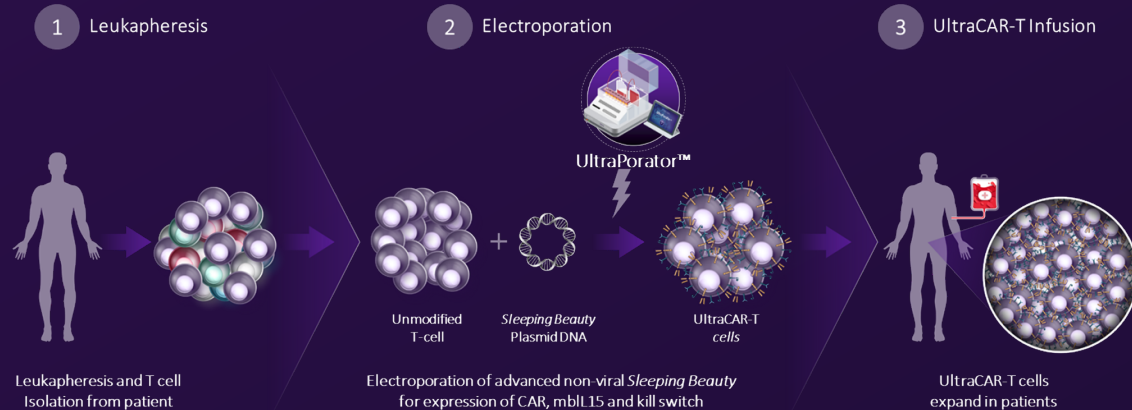
**PRGN-2013 AdenoVerse Immunotherapy (therapeutic HBV vaccine): Initiated IND-enabling studies**



**AG019 ActoBiotics: Completed the Phase 1b/2a clinical trial and presented positive data from the Phase 1b/2a trial in Type 1 diabetes**

## PRGN-3006 TARGETS CD33

- Non-viral system to simultaneously express CD33 CAR, mbIL15 and kill switch
- Overnight, decentralized manufacturing process
- CD33 is overexpressed on AML blasts and leukemic stem cells
- 85-90% of AML patients show expression of CD33 on blast cells<sup>1</sup>
- Minimal expression outside of hematopoietic system



<sup>1</sup>Molica M et al., Cancers 2021

## DISEASE SNAPSHOT



### HIGH UNMET NEED

5-year survival  
as low as 5%  
for AML patients  
over 65<sup>3</sup>

>11K estimated  
deaths from AML  
in 2021<sup>1</sup>



>20K US

Newly  
diagnosed  
AML patients  
per year<sup>1</sup>

>10K US

Newly  
diagnosed  
MDS patients  
per year<sup>2</sup>

<sup>1</sup>American Cancer Society. Key Statistics for Acute Myeloid Leukemia (AML).

<sup>2</sup>American Cancer Society. Key Statistics for Myelodysplastic Syndromes.

<sup>3</sup>Thein, M., et al., Outcome of older patients with acute myeloid leukemia: an analysis of SEER data over 3 decades. Cancer, 2013. 119(15): p.2720-7.

<sup>4</sup>Dohner H, et al., Blood (2010);115:453-474.

<sup>5</sup>Burnett A, et al., J Clin Oncol (2011);29:487-494

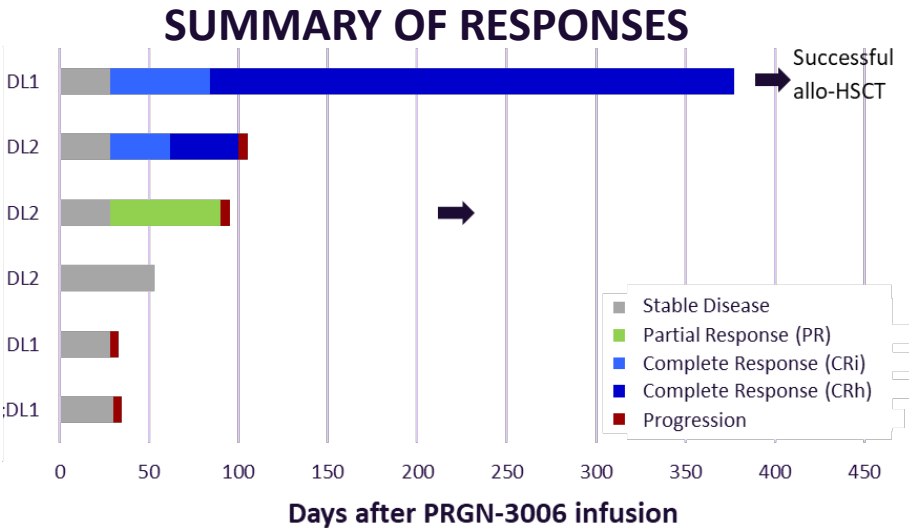
PRGN-3006 PHASE 1/1B CLINICAL TRIAL: CURRENT STATUS

- Completed enrollment in Dose Level 3 of the lymphodepletion cohort
  - Follow-up ongoing
- Excellent safety profile with or without lymphodepletion
  - No DLTs and no neurotoxicity
- Excellent dose-dependent expansion and persistence with or without lymphodepletion in patients
- Results demonstrate feasibility of overnight, decentralized UltraCAR-T manufacturing
- Objective Response Rate (ORR) of 50% in patients treated at the two lowest dose levels in the lymphodepletion cohort

ANTICIPATED 2022 MILESTONES

- Initiate a multicenter expansion phase at Dose Level 3 with lymphodepletion (1H 2022)
- Incorporate PRGN-3006-repeat dosing in the clinical trial
- Present additional Phase 1/1b trial data

COHORT 2 (LYMPHODEPLETION)



Dose Level (DL)	DL1 (N=3)	DL2 (N=3)
Dose Range	>3x10 <sup>4</sup> to ≤1x10 <sup>5</sup> /kg	>1x10 <sup>5</sup> to ≤ 3x10 <sup>5</sup> /kg
Total UltraCAR-T Dose Administered	4.4 – 10 x 10 <sup>6</sup>	18 - 28 x 10 <sup>6</sup>
ORR (%)	33%	67%

Sallman DA et al., Abstract # 825, 63<sup>rd</sup> American Society of Hematology Annual Meeting and Exposition

## OVARIAN CANCER

- Ovarian cancer is the most lethal of the gynecologic malignancies<sup>4</sup>



### HIGH UNMET NEED

Stage IV survival  
as low as 20%<sup>3</sup>



300K WW/22K US

Newly diagnosed  
patients per year<sup>1, 2</sup>

## PRGN-3005 TARGETS UNSHED PORTION OF MUC16

- Non-viral system to simultaneously express MUC16 CAR, mbIL15 and kill switch
- Overnight, decentralized manufacturing process
- MUC16 is overexpressed on >80% of ovarian tumors<sup>5</sup>
- Limited expression found on healthy tissues
- Initial target is advanced stage platinum resistant ovarian cancer

## MUC16 IS OVEREXPRESSED IN VARIOUS SOLID TUMORS

MUC16 expression (% patients)<sup>6</sup>



Ovarian  
Cancer  
Addressable Patient  
Population:  
24,000



Breast  
Cancer  
Addressable Patient  
Population:  
117,000



Pancreatic  
Cancer  
Addressable Patient  
Population:  
33,000



Endometrial  
Cancer  
Addressable Patient  
Population:  
42,000



Lung  
Cancer  
Addressable Patient  
Population:  
144,000

<sup>1</sup>World Health Organization, International Agency for Research on Cancer, Global Cancer Observatory. Cancer Today, Estimated number of new cases in 2018, worldwide, both sexes, all ages.

<sup>2</sup>American Cancer Society Ovarian Cancer Special Section.

<sup>3</sup>American Cancer Society. Survival Rates for Ovarian Cancer, by Stage.

<sup>4</sup>Giannone G. et al., AnnTransl Med (2019).

<sup>5</sup>Suh H, et al., Chemo Open Access (2017)

<sup>6</sup>Human Protein Atlas MUC16 Protein Expression Summary



# PRGN-3005 Phase 1/1b Clinical Trial: Interim Data Demonstrate Excellent Safety and Clinical Activity in Ovarian Cancer Patients

## PRGN-3005 PHASE 1/1B CLINICAL TRIAL: CURRENT STATUS

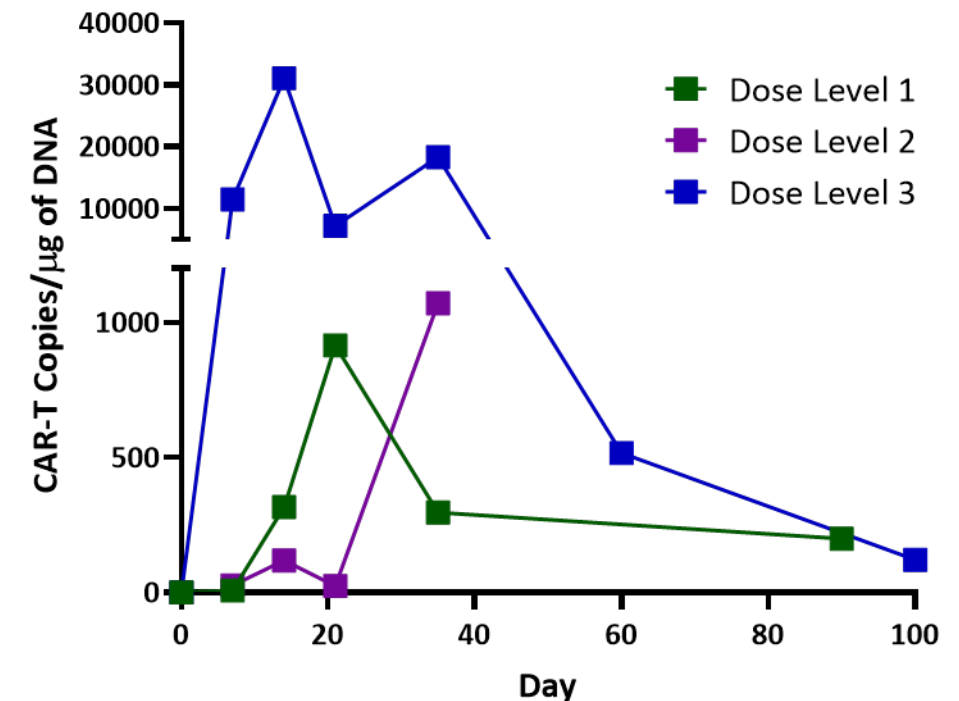
- Completed enrollment in Dose Level 3 of the intravenous (IV) and IP arm
  - Follow-up ongoing
- Excellent safety profile in both the IP and IV arms
  - No DLTs or no neurotoxicity
- Excellent dose-dependent expansion and persistence

## ANTICIPATED 2022 MILESTONES

- Incorporate lymphodepletion at Dose Level 3 of IV arm. FDA clearance received
- Incorporate redosing and initiate multicenter expansion phase

## INTRAPERITONEAL (IP) ARM

### PRGN-3005 EXPANSION IN BLOOD



Limit of quantification: 50 CAR-T copies/μg  
N=1-4 subjects at each time point

Source: Precigen R&D Day Presentation, November 4, 2021

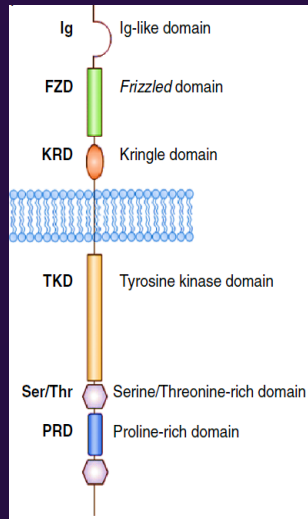


# PRGN-3007 UltraCAR-T: IND Approved to Initiate Phase 1/1b Study in ROR1+ Hematological and Solid Tumors

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## ROR1: AN ATTRACTIVE TARGET FOR HEMATOLOGICAL & SOLID TUMORS

- ROR1 expression contributes to tumor cell growth and survival
- ROR1 is overexpressed in chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL), and acute lymphoblastic leukemia (ALL)<sup>1,2</sup>
- ROR1 is overexpressed in triple negative breast cancer (TNBC), pancreatic cancer, ovarian cancer, and lung adenocarcinomas<sup>1,2</sup>
- Minimal expression on normal adult tissues

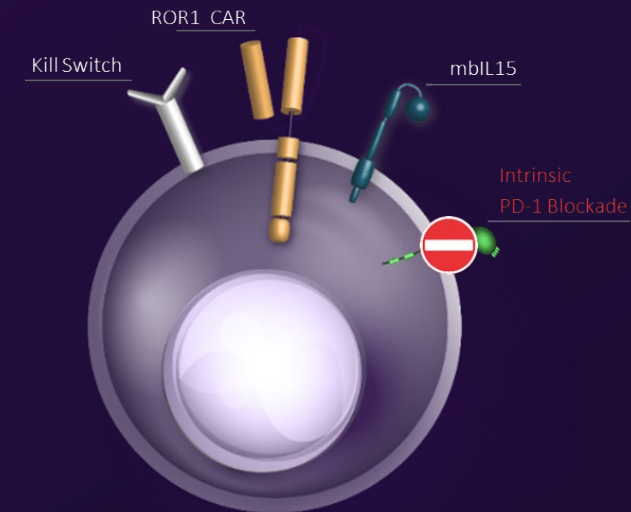


ROR1

Borcherding N, et al, 2014. *Protein & cell* 5:496-502

## PRGN-3007: ROR1 CAR-T WITH INTRINSIC PD-1 INHIBITION

- ROR1 CAR to target various hematologic and solid tumors
- mbIL15 to improve *in vivo* expansion and persistence
- Kill switch to improve safety profile
- Intrinsic downregulation of PD-1 on UltraCAR-T cells to avoid systemic PD-1 blockade



PRGN-3007 UltraCAR-T

<sup>1</sup>Balakrishnan et al. *Clin Cancer Res* 2017;23:3061-3071

<sup>2</sup>Zhang et al, 2012. <http://dx.doi.org/10.1016/j.ajpath.2012.08.024>

INITIATION OF DOSING IN THE PHASE 1 STUDY ANTICIPATED IN 2022

# PRGN-2012: Investigational Off-the-shelf AdenoVerse Immunotherapy for Recurrent Respiratory Papillomatosis (RRP)

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## RRP IS CAUSED BY HPV6 OR HPV11 INFECTION

- A rare disease in which benign tumors called papillomas grow in the respiratory tract
- Symptoms include hoarse voice, difficulty sleeping and swallowing, chronic coughing, or breathing problems
- Affects both children and adults

## DISEASE SNAPSHOT



### HIGH UNMET NEED

No current therapeutic treatment



### 20K Active Cases in US<sup>6</sup>

4 PER 100K

Incidence of RRP in children<sup>1-4</sup>

2-3 PER 100K

Incidence of RRP in adults<sup>5</sup>

<sup>1</sup>Derkay and Wiatrak 2008, National Organization for Rare Disorders 2019

<sup>2</sup>Armstrong, Derkay et al. 1999

<sup>3</sup>Hermann, Pontes et al. 2012

<sup>4</sup>Seedat 2020

<sup>5</sup>National Organization for Rare Disorders 2019

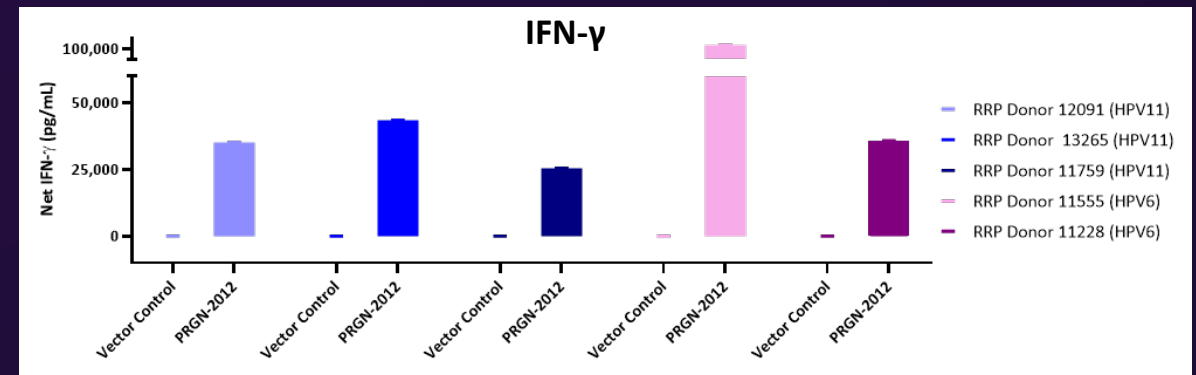
<sup>6</sup>RRP Foundation: <http://www.rrp.org/whatisRRP.html>

<sup>7</sup>Rodriguez-Garcia A. et al., Front. Immunol., 2020

## PRGN-2012 ANTIGEN DESIGN TO TARGET HPV6/11

- Gorilla adenoviral vector with ability for repeat injections
- Antigen designed to induce a robust T cell mediated immune response against HPV6/11
- Orphan Drug Designation (ODD) granted by the FDA

## PRGN-2012 INDUCES ROBUST HPV6 AND HPV11-SPECIFIC T CELL RESPONSE IN RRP PATIENT SAMPLES *IN VITRO*



# PRGN-2012 Phase 1 Clinical Trial: Initial Data Demonstrate Excellent Safety and Response in RRP Patients

## PRGN-2012 PHASE 1 CLINICAL TRIAL: CURRENT STATUS

- **Completed enrollment in the Phase 1 expansion cohort**
  - 15 RRP patients enrolled to date
- Excellent safety profile with repeated administrations of PRGN-2012
  - No DLTs or serious adverse event
- Neutralizing antibody data support repeated administrations of PRGN-2012
- Preliminary data shows very encouraging response in RRP patients, including fewer surgical interventions following PRGN-2012 treatment

## ANTICIPATED 2022 MILESTONES

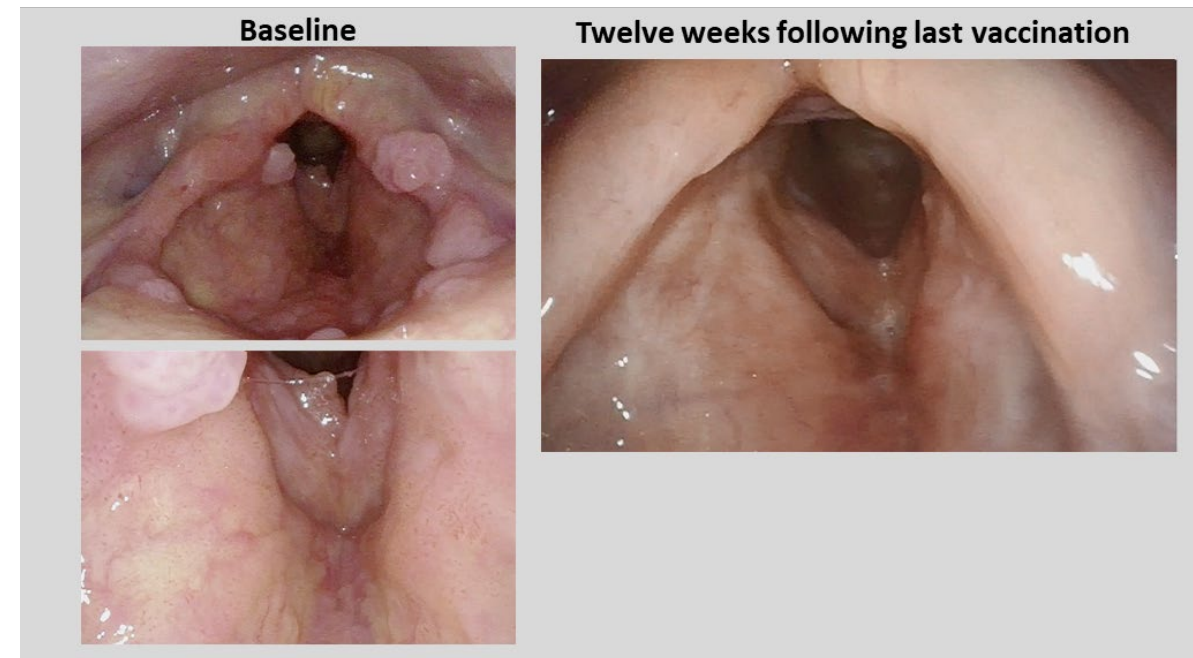
- Seek FDA guidance on rapid regulatory strategy for PRGN-2012 in RRP given significant unmet patient need
- Present additional Phase 1 expansion cohort clinical trial data (2H 2022)

## CASE STUDY: SUBJECT 5 (DOSE LEVEL 1)

- **Required surgery once every 6 weeks for 3 years** prior to enrollment
- Patient received 4 vaccinations of PRGN-2012

### Post treatment:

- No detectable disease present 18 weeks since the last surgery (12 weeks after treatment completion)



## PRGN-2009: MULTI-EPITOPE ANTIGEN DESIGN TO TARGET HPV16/18

- Gorilla adenoviral vector with ability for repeat injections
- Multi-epitope antigen design to induce a robust immune response against HPV16/18



Multi-epitope antigen design

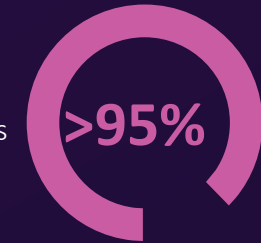
## HPV-ASSOCIATED CANCERS

- HPV infections account for 5% of all cancers<sup>1</sup>
- Globally 690,000 new cancer cases attributable to HPV infections per year<sup>2</sup>



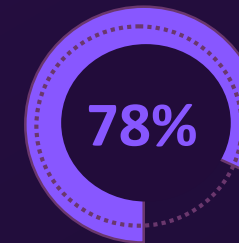
### Cervical Cancer

Addressable New Cases Per Year:  
**570,000**



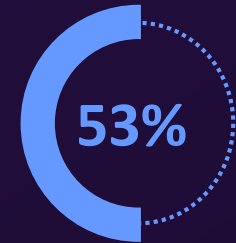
### Anal Cancer

Addressable New Cases Per Year:  
**29,000**



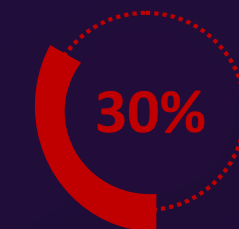
### Vaginal Cancer

Addressable Patient Population:  
**14,000**



### Penile Cancer

Addressable New Cases Per Year:  
**18,000**



### Oropharyngeal Cancer

Addressable New Cases Per Year:  
**42,000**



### Vulvar Cancer

Addressable New Cases Per Year:  
**11,000**

# PRGN-2009 Phase 1 Clinical Trial: Interim Data Demonstrate Excellent Safety and Response in HPV-associated Cancer Patients

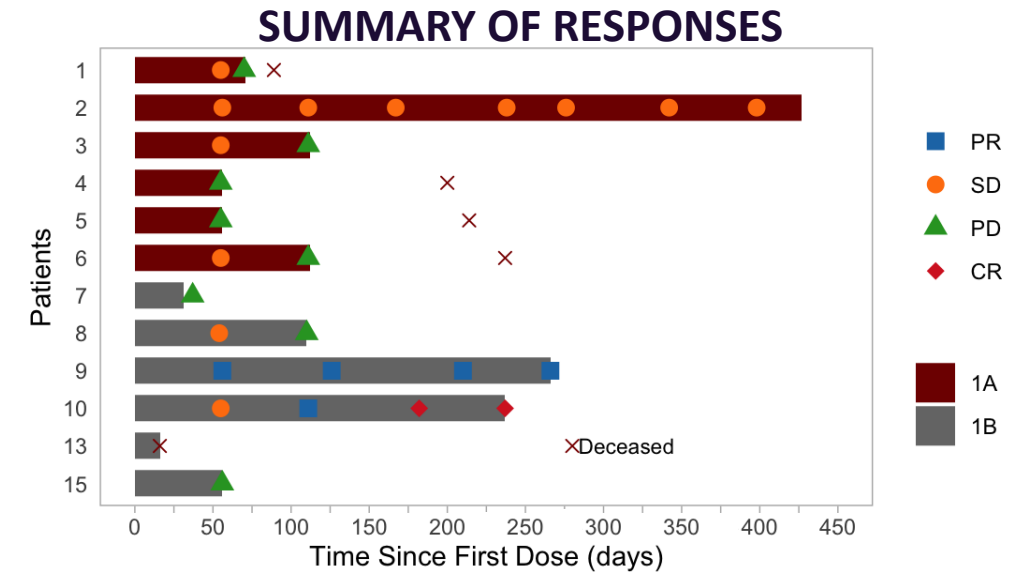
## PRGN-2009 PHASE 1/2 CLINICAL TRIAL: CURRENT STATUS

- Completed enrollment in the Phase 1 monotherapy arm
- Enrollment ongoing in the Phase 1 combination (M7824) arm
- Enrollment in the Phase 2 monotherapy arm in newly diagnosed OPSCC patients is ongoing
- Excellent safety profile with repeated administrations of PRGN-2009 with no DLTs
- Neutralizing antibody data support repeated administrations of PRGN-2009
- Increase in HPV16 and/or HPV18 specific immune response with administrations of PRGN-2009
- Objective Response Rate (ORR) of 40% and Disease Control Rate (DCR) of 60% observed in the combination arm

## ANTICIPATED 2022 MILESTONES

- Seek FDA guidance on a rapid regulatory strategy for PRGN-2009 given interim results and significant unmet patient need
- Initiate a Phase 2 study in advanced HPV-associated cancer in combination with an approved anti-PD1 checkpoint inhibitor

## PHASE 1 CLINICAL TRIAL



	PRGN-2009 Monotherapy (Arm 1A)	PRGN-2009 Combination (Arm 1B)
<b>Disease Control Rate (DCR) at first restaging</b>	<b>50% (3/6)</b>	<b>60% (3/5)</b>
<b>Objective Response Rate (ORR)</b>	<b>0% (0/6)</b>	<b>40% (2/5)</b>

Source: Precigen R&D Day Presentation, November 4, 2021



# AG019 Phase 1b/2a Clinical Trial: Encouraging Stabilization of C-peptide and HbA1c/IDAA1c in Type 1 Diabetes Patients

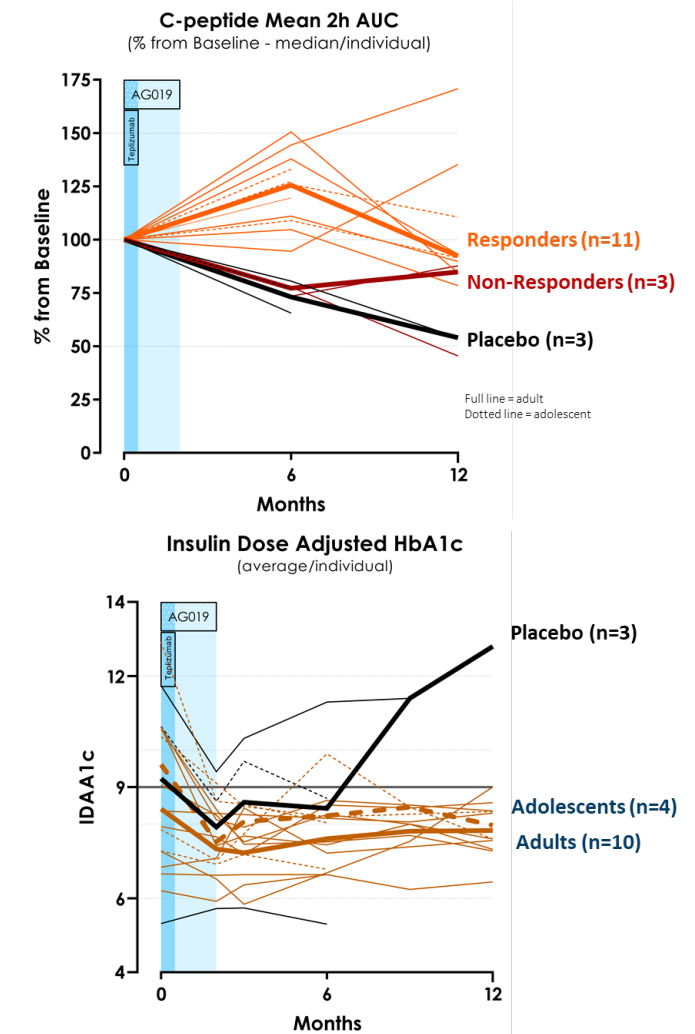
## AG019 : CURRENT STATUS AND NEXT STEPS

- Completed Phase 1b/2a clinical trial of AG019 monotherapy and in combination with teplizumab
- Primary endpoint assessing safety and tolerability met for both Phase 1b and Phase 2a
- Excellent safety profile as monotherapy or in combination No serious adverse events were reported
- AG019, as monotherapy and in combination showed:
  - Stabilization of C-peptide levels, a biomarker for T1D disease progression
  - Stabilization of HbA1c and IDAA1c levels, important indicators of long-term glycemic control T1D patients
- AG019 induced antigen-specific tolerance in conjunction with the reduction of disease-specific T cell responses

## ANTICIPATED 2022 MILESTONES

- Commercial-grade manufacturing scale-up
- Discussions with FDA and EMA for Phase 2/3 clinical trial design

## AG019 MONOTHERAPY



# Summary





**PRGN-3006 UltraCAR-T: Initiate multicenter expansion cohort in AML. Present additional Phase 1/1b data**



**PRGN-3005 UltraCAR-T: Incorporate lymphodepletion and redosing. Initiate expansion cohort in ovarian cancer**



**PRGN-3007 next gen UltraCAR-T: Initiate dosing in Phase 1 study of ROR1<sup>+</sup> hematological (CLL, MCL, ALL, DLBCL) and solid (TNBC) tumors**



**PRGN-2012 AdenoVerse Immunotherapy: Present additional Phase 1 expansion cohort data. Seek FDA guidance on rapid regulatory strategy in RRP given significant unmet patient need**



**PRGN-2009 AdenoVerse Immunotherapy : Initiate a Phase 2 trial in combination with approved anti-PD1 in advanced HPV-associated cancer. Seek FDA guidance on rapid regulatory strategy**



**AG019 ActoBiotics: Develop regulatory path for Phase 2/3 clinical trial with FDA and EMA**



## PORTFOLIO PRIORITIZATION

- Data-driven approach
- Focus on programs with the potential for rapid paths toward licensure
  - PRGN-2009
  - PRGN-2012
  - PRGN-3005
  - PRGN-3006



## FINANCIAL STRENGTH

- Continue to maintain a strong balance sheet
- Continued fiscal discipline
- Operational efficiency
- Seek strategic non-dilutive funding opportunities



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