Precigen Reports Second Quarter and First Half 2023 Financial Results and Provides Update on Portfolio Prioritization and Capital Allocation Strategies to Extend Projected Cash Runway into 2025

Aug 09, 2023

– The FDA confirmed that the ongoing Phase 1/2 single arm study of PRGN-2012 in RRP will serve as the pivotal study to support accelerated approval and no additional randomized, placebo-controlled trial will be required to support submission of a BLA –

– Company to prioritize portfolio activities to accelerate PRGN-2012 and continue advancement of other key programs, by implementing strategies to reduce clinical costs (e.g., reducing CRO costs without internal R&D headcount reduction) and reduce SG&A costs –

– Completely retired the outstanding balance of convertible notes –

– Cash, cash equivalents, short-term and long-term investments totaled $95.6 million as of June 30, 2023; cash runway projected into 2025 –

GERMANTOWN, Md., Aug. 9, 2023 /PRNewswire/ -- Precigen, Inc. (Nasdaq: PGEN), a biopharmaceutical company specializing in the development of innovative gene and cell therapies to improve the lives of patients, today announced second quarter and first half 2023 financial results and business updates.

"Today we announced that the FDA confirmed the ongoing Phase 1/2 study of PRGN-2012 will serve as the pivotal study to support accelerated approval, an important milestone that brings Precigen a step closer in our transition to a commercial stage company and in realizing our vision of bringing life-changing therapies to patients with unmet medical need. We look forward to working with the FDA to submit a BLA and potentially bringing the first drug therapy for RRP patients. As a result of this exciting milestone, we are prioritizing our portfolio activities to maximize the potential success of PRGN-2012 while continuing to strategically advance other key programs," said Helen Sabzevari, PhD, President and CEO of Precigen. "We have built a strong portfolio based on the AdenoVerse and UltraCAR-T platforms and continue to advance important programs with a focus on agility, efficiency and commercial viability."

"As a result of our capital raise in January, our portfolio prioritization and other cost-saving measures, including completely retiring our convertible notes, the Company's balance sheet is well positioned for the future," said Harry Thomasian Jr., CFO of Precigen. "These measures have enabled us to extend our projected cash runway into 2025, exclusive of non-dilutive strategies, including strategic partnerships, which could extend our cash runway further."

Program Highlights

PRGN-2012 AdenoVerse ™ Immunotherapy in RRP

- The Company announced that the US Food and Drug Administration (FDA) has agreed that the ongoing Phase 1/2 single arm study of the first-in-class investigational PRGN-2012 AdenoVerse™ immunotherapy for the treatment of recurrent respiratory papillomatosis (RRP) will serve as pivotal for the purpose of filing an accelerated approval request for licensure. The FDA also confirmed no additional randomized, placebo-controlled trial will be required to support submission of a biologics license application (BLA). Based on the FDA guidance, the Company also plans to initiate a confirmatory study prior to submission of the BLA.
- PRGN-2012 is an investigational off-the-shelf AdenoVerse immunotherapy designed to elicit immune responses directed against cells infected with human papillomavirus (HPV) 6 or HPV 11 for the treatment of RRP.

- The Company completed enrollment and dosing in the Phase 2 portion of the study (N=23) bringing the total number of enrolled patients to 35 at the recommended Phase 2 dose. Patient follow up is currently ongoing and data collection is anticipated to be completed by the second quarter of 2024.
- The Company announced that the FDA granted Breakthrough Therapy Designation for PRGN-2012 for the treatment of RRP, adding to the existing Orphan Drug Designation. The Breakthrough designation is based on positive Phase 1 clinical data that showed that 50% of adult RRP patients (who had ≥3 surgeries to treat the disease in the year prior treatment) were "surgery-free" (Complete Response) after PRGN-2012 treatment during the 12 month follow-up. All complete responders continue to be surgery-free with a minimum follow-up of 18 months post-treatment.

PRGN 2009 AdenoVerse ™ Immunotherapy in HPV-associated Cancers

- PRGN-2009 is an investigational off-the-shelf AdenoVerse immunotherapy designed to activate the immune system to recognize and target HPV-positive solid tumors.
- The Company completed the Phase 1 study and presented positive Phase 1 clinical data from the monotherapy (N=6) and combination therapy (N=11) arms in patients with recurrent or metastatic HPV-associated cancers at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting. PRGN-2009 was safe and well-tolerated with only Grade 1 or 2 treatment related adverse events and resulted in a 30% objective response rate (ORR) in the combination arm in patients with heavily pre-treated HPV-associated cancers that were naïve or resistant to checkpoint blockade with prolonged duration of responses.
- Enrollment was completed in the Phase 2 monotherapy arm with 20 evaluable patients in newly diagnosed oropharyngeal squamous cell carcinoma (OPSCC) patients.
- The Company announced that the FDA has cleared the Investigational New Drug (IND) application to initiate a Phase 2 study of PRGN-2009 in combination with pembrolizumab in patients with recurrent or metastatic cervical cancer. The Phase 2 randomized, open-label, two-arm study will evaluate the efficacy and safety of PRGN-2009 in combination with pembrolizumab versus pembrolizumab monotherapy in patients with recurrent or metastatic cervical cancer who are pembrolizumab resistant.

PRGN-3006 UltraCAR-T® in AML

- PRGN-3006 is an investigational multigenic, autologous chimeric antigen receptor T (CAR-T) cell therapy engineered to express a CAR specifically targeting CD33, membrane bound IL-15 (mbIL15), and a kill switch. The FDA granted Orphan Drug Designation and Fast Track Designation for PRGN-3006 UltraCAR-T for patients with relapsed or refractory acute myeloid leukemia (AML).
- The Company completed the Phase 1 dose escalation study and announced positive data at the 64th American Society of Hematology (ASH) Annual Meeting and Exposition. PRGN-3006 was well-tolerated with no dose-limiting toxicities. A single infusion of autologous PRGN-3006 cells resulted in a 27% ORR in heavily pre-treated relapsed or refractory AML patients infused following lymphodepletion. A single infusion of UltraCAR-T cells with or without lymphodepletion demonstrated robust expansion and persistence in blood and bone marrow and PRGN-3006 infusion with lymphodepletion resulted in a decrease in bone marrow blasts in 60% of heavily pre-treated patients.
- The Phase 1b dose expansion study of PRGN-3006 is ongoing and an interim clinical data presentation is expected in 2024.

PRGN-3005 UltraCAR-T® in Ovarian Cancer

- PRGN-3005 UltraCAR-T is an investigational multigenic, autologous CAR-T cell therapy engineered to express a CAR specifically targeting the unshed portion of MUC16, mbIL15, and a kill switch.
- The Company completed the Phase 1 dose escalation cohorts of the intraperitoneal (IP) and intravenous (IV) arms without lymphodepletion as well as in the lymphodepletion cohort in the IV arm and presented positive Phase 1 clinical data in patients with advanced platinum resistant ovarian cancer at the 2023 ASCO Annual Meeting. PRGN-3005 was well-tolerated with no dose-limiting toxicities, no cytokine release syndrome (CRS) greater than Grade 2, and no neurotoxicity. PRGN-3005 cells demonstrated expansion and persistence when delivered via either IP or IV infusion without lymphodepletion or via IV infusion after lymphodepletion. A single IV infusion following lymphodepletion decreased tumor burden in 67% of the heavily pretreated patients (median of 8 or more prior therapies).
- The Phase 1b dose expansion study of PRGN-3005 is ongoing.

PRGN-3007 UltraCAR-T® in Advanced ROR1+ Hematological and Solid Tumors

- PRGN-3007, based on the next generation of the UltraCAR-T platform, is an investigational multigenic, autologous CAR-T cell therapy engineered to express a CAR targeting receptor tyrosine kinase-like orphan receptor 1 (ROR1), mbIL15, a kill switch, and a novel mechanism for the intrinsic blockade of PD-1 gene expression.
- The Phase 1 dose escalation part of the Phase 1/1b study is ongoing. The target patient population for the Phase 1/1b study includes hematological cancers (chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL), acute

lymphoblastic leukemia (ALL), and diffuse large B-cell lymphoma (DLBCL)) and solid tumors (triple negative breast cancer (TNBC)).

Financial Highlights

- Completely retired the outstanding balance of convertible notes in the second quarter.
- Cash, cash equivalents, short-term and long-term investments totaled $95.6 million as of June 30, 2023.
- Selling, general, and administrative (SG&A) costs decreased versus the prior year, by 27% and 21% for the three and six months ended June 30, 2023, respectively.

Second Quarter 2023 Financial Results Compared to Prior Year Period
Research and development expenses decreased $0.1 million, or 0.7%, compared to the three months ended June 30, 2022. This decrease was primarily driven by reduced spending on preclinical research programs.

SG&A expenses decreased $3.4 million, or 27%, compared to the three months ended June 30, 2022. This decrease was primarily driven by a reduction in professional fees of $2.2 million, due to decreased legal fees associated with certain litigation matters, as well as a $1.1 million reduction in salaries, benefits, and other personnel costs due to reduced head count.

Revenues decreased $1.1 million, or 39%, compared to the three months ended June 30, 2022. This decrease was related to reductions in services performed at Exemplar.

Total other income, net, increased $2.7 million compared to the three months ended June 30, 2023. This was primarily due to reduced interest expense associated with the Company's convertible notes as they were retired in the second quarter of 2023, and increased interest income due to higher interest rates on investments.

Loss from continuing operations was $20.3 million, or $(0.08) per basic and diluted share, compared to loss from continuing operations of $26.1 million, or $(0.13) per basic and diluted share, in Q2 2022.

First Half 2023 Financial Results Compared to Prior Year Period
Research and development expenses increased $0.3 million, or 1.2%, compared to the six months ended June 30, 2022. This increase was primarily driven by a continued prioritization of clinical product candidates, offset by reduced spending on preclinical research programs.

SG&A expenses decreased $5.4 million, or 21%, compared to the six months ended June 30, 2022. This decrease was primarily driven by a reduction in professional fees of $4.2 million, due to decreased legal fees associated with certain litigation matters, as well as a $1.1 million reduction in salaries, benefits, and other personnel costs due to reduced head count.

Revenues decreased $4.8 million, or 57.1%, compared to the six months ended June 30, 2022. This decrease was primarily related to reductions in services performed at Exemplar as well as the recognition of revenue in the first quarter of 2022 related to agreements for which revenue was previously deferred that did not occur in 2023 of $1.0 million at Exemplar.

Total other income, net, increased $5.2 million compared to the six months ended June 30, 2022. This was primarily due to reduced interest expense associated with the Company's convertible notes as they were retired in the second quarter of 2023, and increased interest income due to higher interest rates on investments.

Loss from continuing operations was $43.1 million, or $(0.18) per basic and diluted share, compared to loss from continuing operations of $50.0 million, or $(0.25) per basic and diluted share, in the six months ended June 30, 2022. The 2023 second quarter loss was lower than the 2023 first quarter loss primarily due to continued reductions in SG&A expenses.

Precigen: Advancing Medicine with Precision ™
Precigen (Nasdaq: PGEN) is a dedicated discovery and clinical stage biopharmaceutical company advancing the next generation of gene and cell therapies using precision technology to target the most urgent and intractable diseases in our core therapeutic areas of immuno-oncology, autoimmune disorders, and infectious diseases. Our technologies enable us to find innovative solutions for affordable biotherapeutics in a controlled manner. Precigen operates as an innovation engine progressing a preclinical and clinical pipeline of well-differentiated therapies toward clinical proof-of-concept and commercialization. For more information about Precigen, visit www.precigen.com or follow us on Twitter @Precigen, LinkedIn or YouTube.

UltraCAR-T®
UltraCAR-T is a multigenic autologous CAR-T platform that utilizes Precigen's advanced non-viral *Sleeping Beauty* system to simultaneously express an antigen-specific CAR to specifically target tumor cells, mbIL15 for enhanced *in vivo* expansion and persistence, and a kill switch to conditionally eliminate CAR-T cells for a potentially improved safety profile. Precigen has advanced the UltraCAR-T platform to address the inhibitory tumor microenvironment by incorporating a novel mechanism for intrinsic checkpoint blockade without the need for complex and expensive gene editing techniques. UltraCAR-T investigational therapies are manufactured via Precigen's overnight manufacturing process using the proprietary UltraPorator® electroporation system at the medical center and administered to patients only one day following gene transfer. The overnight UltraCAR-T manufacturing process does not use viral vectors and does not require *ex vivo* activation and expansion of T cells, potentially addressing major limitations of current T cell therapies.

UltraCAR-T® Clinical Program
The UltraCAR-T platform has shifted the autologous CAR-T manufacturing paradigm using an advanced non-viral multigene delivery system and an overnight, decentralized manufacturing process for administration of autologous CAR-T cells one day after gene transfer to reduce vein-to-vein time. Precigen's UltraCAR-T platform is currently under clinical investigation for hematological and solid tumors, including a Phase 1/1b study of PRGN-3005 UltraCAR-T in patients with advanced, recurrent platinum resistant ovarian, fallopian tube or primary peritoneal cancer (NCT03907527), a Phase 1/1b study of PRGN-3006 UltraCAR-T in patients with relapsed or refractory acute myeloid leukemia (AML) or higher risk myelodysplastic syndrome (MDS) (NCT03927261) and a Phase 1/1b study of PRGN-3007 UltraCAR-T incorporating PD-1 checkpoint inhibition in patients with

ROR1-positive (ROR1^{+}) hematologic chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL), acute lymphoblastic leukemia (ALL), diffuse large B-cell lymphoma (DLBCL) and solid tumor triple negative breast cancer (TNBC) malignancies (NCT05694364). PRGN-3006 UltraCAR-T has been granted Orphan Drug Designation and Fast Track Designation in patients with AML by the US Food and Drug Administration (FDA).

UltraCAR-T® Library Approach
Precigen's UltraCAR-T library approach is designed to transform the personalized cell therapy landscape for cancer patients. Precigen's goal is to develop and validate a library of non-viral plasmids to target tumor-associated antigens. Enabled by design and manufacturing advantages of UltraCAR-T, coupled with the capabilities of the UltraPorator® system, Precigen is working to empower cancer centers to deliver personalized, autologous UltraCAR-T treatment with overnight manufacturing to any cancer patient. Based on the patient's cancer indication and biomarker profile, one or more non-viral plasmids would be selected from the library to build a personalized UltraCAR-T treatment. After initial treatment, this approach has the potential to allow for redosing of UltraCAR-T targeting the same or new tumor-associated antigen(s) based on the treatment response and the changes in antigen expression of the patient's tumor. Precigen believes that the combination of the advanced UltraVector® DNA construction platform and the ease of overnight manufacturing gives this library approach a proprietary advantage over traditional T-cell therapies.

UltraPorator®
The UltraPorator system is an exclusive device and proprietary software solution for the scale-up of rapid and cost-effective manufacturing of UltraCAR-T therapies and potentially represents a major advancement over current electroporation devices by significantly reducing the processing time and contamination risk. The UltraPorator device is a high-throughput, semi-closed electroporation system for modifying T cells using Precigen's proprietary non-viral gene transfer technology. UltraPorator is being utilized for clinical manufacturing of Precigen's investigational UltraCAR-T therapies in compliance with current good manufacturing practices.

AdenoVerse ™ Immunotherapy
Precigen's AdenoVerse immunotherapy platform utilizes a library of proprietary adenovectors for the efficient gene delivery of therapeutic effectors, immunomodulators, and vaccine antigens designed to modulate the immune system. Precigen's gorilla adenovectors, part of the AdenoVerse library, have potentially superior performance characteristics as compared to current competition. AdenoVerse immunotherapies have been shown to generate high-level and durable antigen-specific T-cell immune responses as well as an ability to boost these responses via repeat administration. Superior performance characteristics and high yield manufacturing of AdenoVerse vectors leveraging UltraVector® technology allows Precigen to engineer cutting-edge investigational gene therapies to treat complex diseases.

AdenoVerse ™ Immunotherapy Clinical Program
Precigen's AdenoVerse immunotherapy platform is currently under clinical investigation in a Phase 1/2 study of PRGN-2009 AdenoVerse immunotherapy alone or in combination with anti-PDL1/TGF-Beta Trap (bintrafusp alfa) in patients with HPV-associated cancers (NCT04432597), including oropharyngeal squamous cell carcinoma (OPSCC), and a Phase 2 study of PRGN-2012 AdenoVerse immunotherapy in patients with recurrent respiratory papillomatosis (RRP) (NCT04724980). PRGN-2012 has been granted Orphan Drug Designation and Breakthrough Therapy Designation in patients with RRP by the FDA. Additionally, the FDA has cleared the IND to initiate a Phase 2 study of PRGN-2009 AdenoVerse immunotherapy in combination with pembrolizumab in patients with recurrent or metastatic cervical cancer.

For patients interested in enrolling in NCI-led clinical studies, please call NCI's toll-free number 1-800-4-Cancer (1-800-422-6237) (TTY: 1-800-332-8615), email NCIMO_Referrals@mail.nih.gov, and/or visit the website: https://trials.cancer.gov.

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Cautionary Statement Regarding Forward-Looking Statements
Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon the Company's current expectations and projections about future events and generally relate to plans, objectives, and expectations for the development of the Company's business, including the timing and progress of preclinical studies, clinical trials, discovery programs and related milestones, the promise of the Company's portfolio of therapies, and in particular its CAR-T and AdenoVerse therapies. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release. The Company has no obligation to provide any updates to these forward-looking statements even if its expectations change. All forward-looking statements are expressly qualified in their entirety by this cautionary statement. For further information on potential risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Company's most recent Annual Report on Form 10-K and subsequent reports filed with the Securities and Exchange Commission.

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Precigen, Inc. and Subsidiaries

Consolidated Balance Sheets
(Unaudited)

(Amounts in thousands)	June 30, 2023	December 31, 2022
Assets		
Current assets		
Cash and cash equivalents	$ 16,546	$ 4,858
Restricted cash	-	43,339
Short-term investments	71,888	51,092
Receivables		
Trade, net	1,354	978
Other	13,052	12,826
Prepaid expenses and other	2,792	5,066
Total current assets	105,632	118,159
Long-term in investments	7,127	-
Property, plant and equipment, net	6,574	7,329
Intangible assets, net	42,656	44,455
Goodwill	36,966	36,923
Right-of-use assets	7,623	8,086
Other assets	949	1,025
Total assets	$ 207,527	$ 215,977
Liabilities and Shareholders' Equity		
Current liabilities		
Accounts payable	$ 2,510	$ 4,068
Accrued compensation and benefits	4,820	6,377
Other accrued liabilities	3,257	4,997
Settlement and Indemnification Accrual	18,750	18,750
Deferred revenue	15	25
Current portion of long-term debt	-	43,219
Current portion of lease liabilities	1,421	1,209
Total current liabilities	30,773	78,645
Deferred revenue, net of current portion	1,818	1,818
Lease liabilities, net of current portion	6,545	6,992
Deferred tax liabilities	2,181	2,263
Total liabilities	41,317	89,718
Shareholders' equity		
Common stock	-	-
Additional paid-in capital	2,080,348	1,998,314
Accumulated deficit	(1,911,620)	(1,868,567)
Accumulated other comprehensive loss	(2,518)	(3,488)
Total shareholders' equity	166,210	126,259
Total liabilities and shareholders' equity	$ 207,527	$ 215,977

Precigen, Inc. and Subsidiaries
Consolidated Statement of Operations
(Unaudited)

	Three months ended		Six months ended	
(Amounts in thousands, except share and per share data)	**June 30, 2023**	**June 30, 2022**	**June 30, 2023**	**June 30, 2022**
Revenues				
Product revenues	$ 324	$ 621	$ 648	$ 1,113
Service revenues	1,438	2,213	2,965	7,146
Other revenues	5	77	5	165
Total revenues	1,767	2,911	3,618	8,424
Operating Expenses				
Cost of products and services	1,697	1,811	3,224	3,505
Research and development	11,874	11,954	24,037	23,755
Selling, general and administrative	9,316	12,670	20,954	26,359
Impairment of goodwill	-	-	-	482
Impairment of other noncurrent assets	-	638	-	638
Total operating expenses	22,887	27,073	48,215	54,739

Operating loss	(21,120)	(24,162)	(44,597)	(46,315)
Other Expense, Net				
Interest expense	(136)	(2,063)	(460)	(4,101)
Interest income	828	37	1,460	75
Other income, net	44	40	424	238
Total other income (expense), net	736	(1,986)	1,424	(3,788)
Equity in net loss of affiliates	-	-	-	(1)
Loss from continuing operations before income taxes	(20,384)	(26,148)	(43,173)	(50,104)
Income tax benefit	65	89	120	147
Loss from continuing operations	$ (20,319)	$ (26,059)	$ (43,053)	$ (49,957)
Income from discontinued operations, net of income taxes	-	8,424	-	13,071
Net loss	$ (20,319)	$ (17,635)	$ (43,053)	$ (36,886)
Net Loss per share				
Net loss from continuing operations per share, basic and diluted	$ (0.08)	$ (0.13)	$ (0.18)	$ (0.25)
Net income from discontinued operations per share, basic and diluted	-	0.04	-	0.07
Net loss per share, basic and diluted	$ (0.08)	$ (0.09)	$ (0.18)	$ (0.18)
Weighted average shares outstanding, basic and diluted	248,003,322	200,461,441	240,307,403	200,047,629

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