



PRECIGEN

Precigen Reports Third Quarter 2023 Financial Results and Progress of Clinical Programs

Nov 09, 2023

- Based on FDA confirmation in August 2023 that the ongoing Phase 1/2 single-arm study of PRGN-2012 in RRP will serve as the pivotal study to support BLA submission under an accelerated approval request, the Company has expedited efforts to attain commercial readiness –
 - Full results of the Phase 1 portion of the ongoing Phase 1/2 study of PRGN-2012 were published in the peer-reviewed journal, *Science Translational Medicine*, and presented in an oral presentation at the ESGCT 30th Annual Congress –
- Enrollment and dosing was completed in the Phase 2 study of PRGN-2012 in RRP; Phase 2 study completion expected in the second quarter of 2024 –
- Cash, cash equivalents, short-term and long-term investments totaled \$79.0 million as of September 30, 2023; cash runway projected into 2025 –
- Continued focus on cost containment resulted in a reduction in SG&A costs of 17% for the nine months ended September 30, 2023, compared to the prior year over the same period –

GERMANTOWN, Md., Nov. 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 third quarter 2023 financial results and progress of clinical programs.



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ADVANCING MEDICINE WITH PRECISION

"Precigen has made tremendous progress in reducing our operating costs and we are actively reprioritizing our programs to enable commercial readiness for our lead asset, PRGN-2012. We anticipate completing the PRGN-2012 Phase 2 study in the second quarter of 2024 and, given the FDA's guidance in August 2023 that the ongoing Phase 1/2 study of PRGN-2012 will serve as the pivotal study to support an accelerated approval request, we are working to expedite the submission of a BLA as quickly as possible," said Helen Sabzevari, PhD, President and CEO of Precigen. "We have recently published exciting new data for both of the Company's core platforms, AdenoVerse and UltraCAR-T, including presentations at the ESGCT 30th Annual Congress for PRGN-3007 UltraCAR-T and PRGN-2012 AdenoVerse immunotherapy, and publication of a manuscript in *Science Translational Medicine* that includes full Phase 1 data from the PRGN-2012 clinical study. Each publication builds the body of clinical evidence for the potential of our innovative therapeutic platforms in meeting unmet medical needs for patients."

Program Highlights

PRGN-2012 AdenoVerse™ Immunotherapy in RRP

- 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 recurrent respiratory papillomatosis (RRP). The US Food and Drug Administration (FDA) has granted [Breakthrough Therapy Designation](#) and [Orphan Drug Designation](#) for PRGN-2012 for the treatment of RRP.
- The Company announced that the FDA has agreed that the [ongoing Phase 1/2 \(NCT04724980\) single-arm study will serve as pivotal for the purpose of filing an accelerated approval request for licensure](#). Based on this FDA guidance, the Company plans to initiate a confirmatory study prior to submission of the biologics license application (BLA).
- The Company presented positive Phase 1 data showing clinical benefit and enhanced T-cell responses with repeated administration from the ongoing Phase 1/2 single-arm study at the European Society of Gene & Cell Therapy (ESGCT)

30th Annual Congress in an oral presentation (Abstract# OR04) titled, "*Significant clinical benefit and enhanced T-cell responses with repeated administration of PRGN-2012, a novel gorilla adenoviral vector based immunotherapy, in adult patients with severe recurrent respiratory papillomatosis.*"

- The presentation included full results of the Phase 1 study and add to the previously presented data for PRGN-2012 which showed significant response in RRP patients with 50% of patients in Complete Response, requiring no post-treatment surgeries, following PRGN-2012 treatment at Dose Level 2 with a favorable safety profile, no dose-limiting toxicities and no treatment-related adverse events (TRAEs) greater than Grade 2.
- Complete Responses are durable and all complete responders remain surgery-free (follow-up range: 18-24 months) after PRGN-2012 treatment completion.
- PRGN-2012 treatment induced robust HPV-specific T cell responses which were correlated with clinical responses in the study.
- Full results of the Phase 1 portion of the ongoing Phase 1/2 study of PRGN-2012 were published in the peer-reviewed journal, *Science Translational Medicine*, a leading publication from the American Association for the Advancement of Science (AAAS), in a manuscript titled, "[The tumor microenvironment state associates with response to HPV therapeutic vaccination in patients with respiratory papillomatosis.](#)"
- Enrollment and dosing in the Phase 2 portion of the study (N=23) is complete bringing the total number of enrolled patients to 35 at Dose Level 2. Patient follow up is currently ongoing and the Phase 2 study is expected to be complete by the second quarter of 2024.

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 (NCT04432597) study and [presented positive Phase 1 clinical data](#) from the monotherapy and combination therapy arms in patients with recurrent or metastatic HPV-associated cancers at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting.
- Enrollment was completed in the Phase 2 monotherapy arm with 20 evaluable patients in newly diagnosed oropharyngeal squamous cell carcinoma (OPSCC) patients. The Phase 2 (NCT05996523) combination arm (PRGN-2009 in combination with pembrolizumab) in OPSCC is enrolling patients.
- The Company plans to initiate a Phase 2 randomized, open-label, two-arm study of PRGN-2009 in combination with pembrolizumab in patients with recurrent or metastatic cervical cancer.

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 (NCT03927261) dose escalation study and [presented positive data](#) at the 64th American Society of Hematology (ASH) Annual Meeting and Exposition.
- 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 (NCT03907527) 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.
- As previously communicated, based on portfolio reprioritization efforts, the Company will not add an extensive number of new sites this year. Instead, a new site will be activated under the Cooperative Research and Development Agreement (CRADA) with the National Cancer Institute (NCI) to continue the advancement of the PRGN-3005 Phase 1b dose expansion study without incurring major clinical/contract research organization (CRO) costs.

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 portion of the Phase 1/1b study is ongoing. The target patient population for the Phase 1/1b study includes ROR1+ advanced hematological and solid tumors.

- The Company presented additional preclinical data (Abstract# P469) for PRGN-3007 at the ESGCT 30th Annual Congress in a poster presentation titled, "*Overnight-manufactured UltraCAR-T® cells with first-in-class miRNA-based PD1 blockade demonstrates enhanced polyfunctionality and sustained cytotoxicity against hematological and solid tumors.*"

Financial Highlights

- Cash, cash equivalents, short-term and long-term investments totaled \$79.0 million as of September 30, 2023.
- Selling, general, and administrative (SG&A) costs decreased versus the prior year, by 9% and 17% for the three and nine months ended September 30, 2023, respectively.

"Following our portfolio reprioritization and other cost-saving measures announced last quarter, Precigen continues to manage the balance sheet to enable rapid progression of our lead assets," said Harry Thomasian Jr., CFO of Precigen. "As we scale-up areas of our business to prepare for commercialization, we are focused on fiscal management and exploring new non-dilutive capital opportunities, including potential strategic partnerships, to maximize and extend our runway."

Third Quarter 2023 Financial Results Compared to Prior Year Period

Research and development expenses decreased \$1.0 million, or 8%, compared to the three months ended September 30, 2022. This decrease was primarily due to continued reprioritization of clinical product candidates.

SG&A expenses decreased \$0.9 million, or 9%, compared to the three months ended September 30, 2022. This decrease was primarily driven by a reduction in professional fees of \$0.6 million, due to decreased legal fees associated with certain litigation matters, and \$0.3 million in decreased insurance related expenses.

Total revenues decreased \$15.3 million, or 92%, compared to the three months ended September 30, 2022. Collaboration and licensing revenues decreased \$14.6 million, or 100%, compared to the three months ended September 30, 2022, primarily due to the prior year period non-cash recognition of revenue related to historical collaboration agreements for which revenue was previously deferred. Product and service revenues decreased \$0.7 million, or 34%, compared to the three months ended September 30, 2022. This decrease is related to reductions in services performed at Exemplar.

Total other income, net, increased \$2.1 million compared to the three months ended September 30, 2022. This is primarily due to \$2.0 million in reduced interest expense associated with the Company's Convertible Notes as they were fully retired in the second quarter of 2023, and \$0.8 million increased interest income due to higher interest rates on investments. This increase was offset by a \$0.9 million gain recorded on the early retirement of a portion of the Convertible Notes in the third quarter of 2022 that did not occur in the third quarter of 2023.

Loss from continuing operations was \$19.8 million, or \$(0.08) per basic and diluted share, compared to loss from continuing operations of \$7.6 million, or \$(0.04) per basic and diluted share, in the three months ended September 30, 2022.

First Nine months 2023 Financial Results Compared to Prior Year Period

Research and development expenses decreased \$0.8 million, or 2%, compared to the nine months ended September 30, 2022. This decrease was primarily due to continued reprioritization of clinical product candidates.

SG&A expenses decreased \$6.3 million, or 17%, compared to the nine months ended September 30, 2022. This decrease was primarily driven by a reduction in professional fees of \$4.8 million, due to decreased legal fees associated with certain litigation matters, as well as a \$1.2 million reduction in salaries, benefits, and other personnel costs due to reduced head count, and \$0.3 million in decreased insurance related expenses.

Total revenues decreased \$20.1 million, or 80%, from the nine months ended September 30, 2022. Collaboration and licensing revenues decreased \$14.6 million or 100% from the nine months ended September 2022, primarily due to the prior year period non-cash recognition of revenue related to historical collaboration agreements for which revenue was previously deferred. Product and services revenues decreased \$5.4 million, or 52%, from the nine months ended September 30, 2022. This decrease 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 \$7.3 million compared to the nine months ended September 30, 2022. This is primarily due to \$5.7 million reduced interest expense associated with the Convertible Notes as they were retired in the second quarter of 2023, and \$2.1 million increased interest income due to higher interest rates on the Company's investments. This increase was offset by \$0.8 million reduction in the gain recorded on the early retirement of a portion of the Convertible Notes in 2023 compared to 2022.

Loss from continuing operations was \$62.8 million, or \$(0.26) per basic and diluted share, compared to loss from continuing operations of \$57.6 million, or \$(0.29) per basic and diluted share, in the nine months ended September 30, 2022.

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 X @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, mβIL15 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 patient's 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

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⁺) chronic lymphocytic leukemia (CLL), mantle cell lymphoma (MCL), acute lymphoblastic leukemia (ALL), diffuse large B-cell lymphoma (DLBCL) and triple negative breast cancer (TNBC) ([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 medical 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 an anti-PDL1/TGF-Beta Trap (bintrafusp alfa) in patients with HPV-associated cancers ([NCT04432597](#)), including the Phase 2 combination arm (PRGN-2009 in combination with pembrolizumab) in newly diagnosed patients with HPV-associated oropharyngeal squamous cell carcinoma (OPSCC) ([NCT05996523](#)), and a Phase 1/2 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>.

Trademarks

Precigen, UltraCAR-T, UltraPorator, AdenoVerse, UltraVector, Advancing Medicine with Precision are trademarks of Precigen and/or its affiliates. Other names may be trademarks of their respective owners.

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)	September 30, 2023		December 31, 2022	
Assets				
Current assets				
Cash and cash equivalents	\$	10,076	\$	4,858
Restricted cash		-		43,339
Short-term investments		63,679		51,092
Receivables				
Trade, net		988		978
Other		13,117		12,826
Prepaid expenses and other		5,128		5,066
Total current assets		92,988		118,159
Long-term investments		5,271		-
Property, plant and equipment, net		7,115		7,329
Intangible assets, net		40,426		44,455
Goodwill		36,894		36,923
Right-of-use assets		7,197		8,086
Other assets		797		1,025
Total assets	\$	190,688	\$	215,977
Liabilities and Shareholders' Equity				
Current liabilities				
Accounts payable	\$	2,351	\$	4,068
Accrued compensation and benefits		6,621		6,377
Other accrued liabilities		4,119		4,997
Settlement and indemnification accruals		18,075		18,750
Deferred revenue		509		25
Current portion of long-term debt		-		43,219
Current portion of lease liabilities		1,200		1,209
Total current liabilities		32,875		78,645
Deferred revenue, net of current portion		1,818		1,818
Lease liabilities, net of current portion		6,192		6,992
Deferred tax liabilities		2,125		2,263
Total liabilities		43,010		89,718
Shareholders' equity				
Common stock		-		-
Additional paid-in capital		2,082,654		1,998,314
Accumulated deficit		(1,931,415)		(1,868,567)
Accumulated other comprehensive loss		(3,561)		(3,488)
Total shareholders' equity		147,678		126,259
Total liabilities and shareholders' equity	\$	190,688	\$	215,977

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

(Amounts in thousands, except share and per share data)	Three months ended		Nine months ended	
	September 30, 2023	September 30, 2022	September 30, 2023	September 30, 2022
Revenues				
Collaboration and licensing revenues	\$	-	\$	14,561
Product revenues		82		342
			\$	730
			\$	1,455

Service revenues	1,296	1,750	4,261	8,896
Other revenues	1	69	6	234
Total revenues	1,379	16,722	4,997	25,146
Operating Expenses				
Cost of products and services	1,537	1,577	4,761	5,082
Research and development	11,583	12,622	35,620	36,377
Selling, general and administrative	9,196	10,137	30,150	36,496
Impairment of goodwill	-	-	-	482
Impairment of other noncurrent assets	-	-	-	638
Total operating expenses	22,316	24,336	70,531	79,075
Operating loss	(20,937)	(7,614)	(65,534)	(53,929)
Other income (Expense), Net				
Interest expense	(1)	(2,036)	(461)	(6,137)
Interest income	856	56	2,316	131
Other income, net	281	1,038	705	1,276
Total other income (expense), net	1,136	(942)	2,560	(4,730)
Equity in net income (loss) of affiliates	-	862	-	861
Loss from continuing operations before income taxes	(19,801)	(7,694)	(62,974)	(57,798)
Income tax benefit	6	50	126	197
Loss from continuing operations	\$ (19,795)	\$ (7,644)	\$ (62,848)	\$ (57,601)
Income from discontinued operations, net of income taxes	-	95,023	-	108,094
Net (loss) income	\$ (19,795)	\$ 87,379	\$ (62,848)	\$ 50,493
Net (loss) income per share				
Net loss from continuing operations per share, basic and diluted	\$ (0.08)	\$ (0.04)	\$ (0.26)	\$ (0.29)
Net income from discontinued operations per share, basic and diluted	-	0.48	-	0.54
Net (loss) income per share, basic and diluted	\$ (0.08)	\$ 0.44	\$ (0.26)	\$ 0.25
Weighted average shares outstanding, basic and diluted	248,520,724	200,670,590	243,075,262	200,256,046

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