

April 29, 2025



# **U.S. FDA Approves ZEVASKYN™ (prademagene zamikeracel), the First and Only Cell-Based Gene Therapy for Patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)**

- *ZEVASKYN fills a critical need for people living with RDEB, a debilitating dermatological condition with no cure -*
- *Approval based on the pivotal Phase 3 VIITAL™ study, showing ZEVASKYN resulted in significant wound healing and pain reduction after a single treatment with a favorable safety profile -*
- *ZEVASKYN to be available through Qualified Treatment Centers beginning in 3Q 2025 -*
- *Abeona Assist™ patient services program offers personalized support for eligible patients and families throughout their treatment journey with ZEVASKYN -*
- *Rare Pediatric Disease Priority Review Voucher (PRV) granted by FDA -*
- *Abeona Therapeutics® to host conference call today, Tuesday, April 29, 2025, at 8:00 a.m. ET -*

CLEVELAND, April 29, 2025 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced the U.S. Food and Drug Administration (FDA) has approved ZEVASKYN™ (pronounced as 'ZEE-vah-skin') (prademagene zamikeracel) gene-modified cellular sheets, also known as pz-cel, as the first and only autologous cell-based gene therapy for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB), a serious and debilitating genetic skin disease. There is no cure for RDEB and ZEVASKYN is the only FDA-approved product to treat RDEB wounds with a single application.

"Today's approval of ZEVASKYN represents a pivotal moment in the treatment of RDEB, answering the call of people living with the clinical, economic, and human impact of this devastating disease," said Vish Seshadri, Ph.D., M.B.A., Chief Executive Officer of Abeona. "We have heard from the RDEB community that there is a persistent unmet need to reliably address RDEB wounds, especially those that are chronic and prone to infection. Through a single surgical application, ZEVASKYN can now offer people with RDEB the opportunity for wound healing and pain reduction in even the most severe wounds, as evidenced by the results from our pivotal Phase 3 study."

## **First-of-its-kind gene therapy with robust body of clinical evidence**

The FDA approval of ZEVASKYN is based on the pivotal Phase 3 VIITAL™ study (NCT04227106), a multi-center, randomized, inpatient-controlled trial that met its two co-primary efficacy endpoints demonstrating statistically significant healing of 50 percent or more from baseline in large chronic RDEB wounds, and pain reduction from baseline as assessed by the Wong-Baker FACES scale, as evaluated at six months after treatment.

Across 43 large and chronic wounds treated with a single application of ZEVASKYN, 81 percent of wounds showed 50 percent or more healing ( $P < 0.0001$ ) as evaluated at six months, compared to 16 percent in 43 matched control wounds treated with standard of care. The most common adverse events were observed in fewer than five percent of patients and included procedural pain and itch.

“ZEVASKYN was well-tolerated and efficacious in clinical studies, providing clinically meaningful improvements in wound healing, pain reduction, and other associated symptoms in large chronic RDEB wounds after a single application,” said Jean Tang, M.D., Ph.D., professor of dermatology and lead principal investigator of the VIITAL™ study. “In the completed Phase 1/2a study of ZEVASKYN, we have observed wound healing and pain reduction that have lasted for years after a single application. Today we can celebrate the availability of an exciting new therapeutic option made possible by the incredible courage of patients and families who participated in these clinical studies.”

In the Phase 1/2a study of ZEVASKYN (NCT01263379), a single center, open label study in 38 chronic wounds across 7 patients showed that a single surgical application of ZEVASKYN was associated with long-term improvement at treated sites over a median follow-up of 6.9 years; range 4 to 8 years.

“After many years of work, it is great to see this FDA approval of ZEVASKYN. The EB patients deserve all that we can do for them,” said M. Peter Marinkovich, M.D., associate professor of dermatology and co-principal investigator of the VIITAL™ study.

“Based on the strength of our data across clinical trials, we are confident in ZEVASKYN’s ability to deliver long-term results after a single treatment application,” said Madhav Vasanthavada, Ph.D., M.B.A., Chief Commercial Officer of Abeona. “We are committed to working closely with both commercial and government payers on outcome-based agreements that stand behind the promise of ZEVASKYN for patients, and expedite access.”

Across both clinical studies, ZEVASKYN was well-tolerated with no treatment-related serious adverse events observed to date.

Anna L. Bruckner, MD, Co-Director of the EB Clinic at Children’s Hospital of Colorado and Professor of Dermatology, University of Colorado School of Medicine, said, “The FDA approval of ZEVASKYN marks a monumental step forward for individuals living with RDEB and their families, offering a much-needed, long-lasting treatment option for this devastating condition and providing hope for improved quality of life for these patients.”

Amy Paller, M.D., pediatric dermatologist and clinical researcher, said, “Grafting gene-corrected skin onto chronically open wounds of patients with recessive dystrophic epidermolysis bullosa promises the potential to provide long-term healing of wounds,

reduction in pain and reduced risk of infection. This therapeutic option will nicely complement recently approved topical products.”

Dr. Seshadri added, “We are grateful to the patients, their families, and caregivers for their support of ZEVASKYN. We express our gratitude to debra of America for their unwavering support throughout the development journey, in particular, for their interactions with the FDA in support of ZEVASKYN and on behalf of the EB community that have helped make today’s regulatory approval possible. We are also thankful for the clinical study investigators, study site personnel, and the entire Abeona team for their collective commitment and determination through the development process, and contribution to this milestone achievement. We look forward to providing the RDEB community access to now-approved ZEVASKYN.”

### **Addressing the underlying cause of RDEB**

With mutations in both copies of the *COL7A1* gene that expresses Type VII collagen, people with RDEB have extremely fragile skin characterized by extensive blistering and severe wounds that often cover more than 30 percent of a patient’s body surface, and in some cases up to 80 percent. RDEB wounds cause debilitating pain and systemic complications impacting the length and quality of life. These wounds are difficult to heal, can remain open for years, and many that do close tend to reopen.

ZEVASKYN consists of a patient’s own skin cells (keratinocytes) that have been genetically modified, to produce functional Type VII collagen. ZEVASKYN sheets are surgically applied to the patient’s wounded areas. In a single application of ZEVASKYN, up to 12 credit card-sized sheets can be joined together to cover large areas or applied to multiple distinct wounds, allowing for significant coverage of affected body areas.

Brett Kopelan, M.A., Executive Director of debra of America, the only national advocacy organization that provides all-inclusive care to the epidermolysis bullosa community, and father to Rafi, a teenager with RDEB, said, “I, and the team at debra of America, are very excited about the FDA’s approval of ZEVASKYN. Given that this therapeutic product addresses even the largest, most difficult, and problematic chronic wounds, we believe that the application of ZEVASKYN can significantly increase the quality of life of patients. Furthermore, I believe ZEVASKYN has the potential to transform the day-to-day standard of care for patients who suffer with these large chronic nonhealing wounds that cause significant pain and stress not only for the patient, but also for their caregivers. We are honored to have formed such a close relationship with Abeona over the years and look forward to deepening our partnership by helping ensure there is broad access for the patient population to ZEVASKYN, which I know is their ultimate goal.”

Michael Hund, M.B.A., Chief Executive Officer of EB Research Partnership (EBRP), the largest global organization dedicated to funding research to treat and cure epidermolysis bullosa (EB), said, “The mission of EBRP is to advance commercially sustainable research aimed at treating and ultimately curing EB. We are honored to partner with the entire Abeona team and commend their leadership, determination, and passion to deliver much needed innovative solutions for individuals and their families living with EB. They continue to share our values and commitment to accelerate treatments to the EB community as quickly as possible. Abeona’s development and advancement of ZEVASKYN delivers a landmark moment for the global EB community, and their leadership in gene therapy holds so much

promise to innovate the therapeutic landscape for not only EB, but many other rare diseases and conditions. EBRP is looking forward to collaborating with Abeona to continue to support the EB community.”

### **ZEVASKYN availability in the third quarter of 2025**

ZEVASKYN is expected to be available beginning in the third quarter of 2025 through ZEVASKYN Qualified Treatment Centers (QTCs). The QTCs are well-recognized epidermolysis bullosa treatment centers with cell and gene therapy experience, situated across the U.S. to ensure patients nationwide have access to this important treatment.

Abeona’s comprehensive patient support program, Abeona Assist™, offers personalized support, including helping patients understand their insurance benefits and financial assistance options, and providing travel and logistical assistance for eligible patients. For more information on how to access ZEVASKYN, patients, caregivers, and providers may call Abeona Assist at 1-855-ABEONA-1 (1-855-223-6621) or email [MyNavigator@AbeonaAssist.com](mailto:MyNavigator@AbeonaAssist.com).

“We are grateful to the dedicated scientists whose work over the past decade made the development of ZEVASKYN possible,” said Marissa Perman, MD, Section Chief of Dermatology and Director of the Epidermolysis Bullosa Multidisciplinary Clinic at Children’s Hospital of Philadelphia and paid consultant to Abeona. “Having a new, uniquely differentiated, gene therapy for our patients with RDEB is a significant milestone in helping these special patients live fuller, pain-free and itch-free lives with less wounds. As a physician caring for patients with RDEB, I look forward to the opportunity to see this treatment in our practice.”

Joyce Teng, MD, PhD, professor in dermatology with multiple hospital affiliations, said, “I’m thrilled to celebrate a groundbreaking advancement in therapeutic development for recessive dystrophic EB, a condition that has long needed an innovative solution. ZEVASKYN offers additional hope for patients and families affected by this painful and devastating skin disorder. This milestone is a testament to the dedication of scientists, researchers and medical professionals who have worked tirelessly to bring cutting edge treatments to those in need. It represents a scientific triumph, a profound step toward improving quality of life for individuals affected. We look forward to seeing the impact that this therapy will have on so many lives.”

In connection with the FDA approval, Abeona received a Rare Pediatric Disease Priority Review Voucher (PRV). The Company plans to monetize the PRV.

### **Conference Call Details**

Abeona Therapeutics will host a conference call and webcast with accompanying slides and patient videos today, Tuesday, April 29, 2025, at 8:00 a.m. ET. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 320280 five minutes prior to the start of the call. A live, listen-only webcast and archived replay of the call can be accessed at <https://investors.abeonatherapeutics.com/events>. The archived webcast replay will be available for 30 days following the call.

### **Indication**

ZEVASKYN™ (prademagene zamikeracel) is an autologous cell sheet-based gene therapy indicated for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB).

### **Important Safety Information**

- Serious allergic reactions to ZEVASKYN can occur. Patients should get medical help right away if they experience symptoms like itching, swelling, hives, difficulty breathing, runny nose, watery eyes, or nausea. In rare cases, a severe reaction called anaphylaxis may happen.
- There is a potential risk that treatment with ZEVASKYN may contribute to the development of cancer because of how the therapy works. Patients should be monitored for the rest of their lives to check for any signs of cancer.
- ZEVASKYN is made using human and animal materials. Although these materials are tested before use, the risk of passing on infections cannot be eliminated.
- The most common side effects are pain from the procedure and itching.

This is not a complete list of side effects. Patients should call their care team for medical advice about side effects. Side effects may be reported to Abeona at 1-844-888-2236 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

See full [Prescribing Information](#) starting today, Tuesday, April 29, 2025, at approximately 7:00 a.m. ET.

### **About Recessive Dystrophic Epidermolysis Bullosa**

Recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without a cure, is characterized by severe skin wounds that cause pain and can lead to systemic complications impacting the length and quality of life. People with RDEB have a defect in both copies of the COL7A1 gene, leaving them unable to produce functioning type VII collagen, which is necessary to anchor the dermal and epidermal layers of the skin.

### **About ZEVASKYN™ (prademagene zamikeracel) gene-modified cellular sheets or pz-cel**

ZEVASKYN is the first and only autologous cell sheet-based gene therapy for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB). RDEB is a severe skin disease caused by a defect in both copies of the COL7A1 gene resulting in the inability to produce functional type VII collagen. Without functional type VII collagen and anchoring fibrils, the skin is fragile and blisters easily, leading to wounds that continually open and close, or fail to heal altogether. Patients often have large open wounds that are at a high risk of systemic infection. ZEVASKYN incorporates the functional type VII collagen-producing COL7A1 gene into a patient's own skin cells, ex vivo, using a replication-incompetent retroviral vector to produce functional type VII collagen in treated wounds. ZEVASKYN has demonstrated clinically meaningful wound healing and pain reduction with a single surgical application. For more information, visit [www.ZEVASKYN.com](http://www.ZEVASKYN.com).

### **About Abeona Therapeutics**

Abeona Therapeutics Inc. is a commercial-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's ZEVASKYN™ (prademagene zamikeracel) is the first and only autologous cell-based gene therapy for the treatment of wounds in adults and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB). The Company's fully integrated cell and gene therapy cGMP manufacturing facility in Cleveland, Ohio serves as the manufacturing site for ZEVASKYN commercial production. The Company's development portfolio features adeno-associated virus (AAV)-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. For more information, visit [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com).

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### **Forward-Looking Statements**

*This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. We have attempted to identify forward-looking statements by such terminology as “may,” “will,” “believe,” “anticipate,” “expect,” “intend,” “potential,” and similar words and expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to, our ability to commercialize ZEVASKYN, the therapeutic potential of ZEVASKYN, whether the unmet need and market opportunity for ZEVASKYN are consistent with the Company's expectations, continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with and inspections from the FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; the impact of any changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; and other risks disclosed in the Company's most recent Annual Report on Form 10-K and subsequent periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.*

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