

# Epidermolysis bullosa - Pipeline Insight, 2021

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### **Abstracts**

This report can be delivered to the clients within 48 Hours

DelveInsight's, "Epidermolysis bullosa - Pipeline Insight, 2021," report provides comprehensive insights about 25+ companies and 25+ pipeline drugs in Epidermolysis bullosa pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Epidermolysis bullosa Understanding

Epidermolysis bullosa: Overview

Epidermolysis bullosa (EB) is a genetic skin disorder characterized clinically by blister formation from mechanical trauma. There are four main types with additional sub-types identified. There is a spectrum of severity, and within each type, one may be either mildly or severely affected. EB ranges from being a minor inconvenience requiring modification of some activities, to being completely disabling and, in some cases, fatal. Friction causes blister formation. Blisters can form anywhere on the surface of the skin, within the oral cavity and in more severe forms may also involve the external surface of the eye, as well as the respiratory, gastrointestinal and genitourinary tracts. In some forms of the disease, disfiguring scars and disabling musculoskeletal deformities occur.



'Epidermolysis bullosa - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Epidermolysis bullosa pipeline landscape is provided which includes the disease overview and Epidermolysis bullosa treatment guidelines. The assessment part of the report embraces, in depth Epidermolysis bullosa commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Epidermolysis bullosa collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

# Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Epidermolysis bullosa R&D. The therapies under development are focused on novel approaches to treat/improve Epidermolysis bullosa.

Epidermolysis bullosa Emerging Drugs Chapters

This segment of the Epidermolysis bullosa report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Epidermolysis bullosa Emerging Drugs

EB-101: Abeona Therapeutics

EB-101 is an autologous, gene-corrected cell therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB), a rare connective tissue disorder without an approved treatment in which patients suffer with severe epidermal wounds that impact the length and quality of their lives. People with RDEB have a defect in the COL7A1 gene, leaving them unable to produce Type VII collagen that helps anchor the dermal and epidermal layers of the skin. Treatment with EB-101 involves using gene transfer to deliver COL7A1 genes into an RDEB patient's own skin cells (keratinocytes) and transplanting



them back to the patient to enable normal Type VII collagen expression and skin function. Investigators are currently enrolling the VIITAL™ Study, a pivotal Phase III clinical trial evaluating EB-101 for the treatment of RDEB patients.

D-Fi (FCX-007): Castle Creek Biosciences

D-Fi (dabocemagene autoficel, formerly known as FCX-007), is being developed as a disease-modifying, autologous cell-based gene therapy to address the deficiency of functional type VII collagen protein (COL7) in patients with autosomally recessive or dominant dystrophic epidermolysis bullosa, or DEB. D-Fi is comprised of autologously-derived dermal fibroblasts genetically modified with a lentiviral vector containing the COL7A1 gene, to express COL7. D-Fi is locally administered by injection directly into the papillary dermis of wounds of DEB where the COL7 protein can support the formation of anchoring fibrils in the skin, thereby avoiding systemic treatment. The U. S. Food and Drug Administration has granted Orphan Drug designation to D-Fi for the treatment of Dystrophic Epidermolysis Bullosa, which includes RDEB. In addition, D-Fi has been granted Rare Pediatric Disease designation, Fast Track designation and Regenerative Medicine Advanced Therapy (RMAT) designation by the FDA for treatment of RDEB.

Further product details are provided in the report......

Epidermolysis bullosa: Therapeutic Assessment

This segment of the report provides insights about the different Epidermolysis bullosa drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Epidermolysis bullosa

There are approx. 25+ key companies which are developing the therapies for Epidermolysis bullosa. The companies which have their Epidermolysis bullosa drug candidates in the most advanced stage, i.e. phase III include, Abeona Therapeutics.

Phases



DelveInsight's report covers around 25+ products under different phases of clinical development like

Late stage products (Phase III)
Mid-stage products (Phase II)
Early-stage product (Phase I) along with the details of
Pre-clinical and Discovery stage candidates
Discontinued & Inactive candidates
Route of Administration
Epidermolysis bullosa pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as
Oral
Parenteral
Intravenous
Subcutaneous
Topical.
Molecule Type
Products have been categorized under various Molecule types such as
Monoclonal Antibody
Peptides



Polymer
Small molecule
Gene therapy
Product Type
Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.
Epidermolysis bullosa: Pipeline Development Activities
The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Epidermolysis bullosa therapeutic drugs key players involved in developing key drugs.
Pipeline Development Activities
The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Epidermolysis bullosa drugs.
Epidermolysis bullosa Report Insights
Epidermolysis bullosa Pipeline Analysis
Therapeutic Assessment
Unmet Needs
Impact of Drugs

Epidermolysis bullosa - Pipeline Insight, 2021

Epidermolysis bullosa Report Assessment

Pipeline Product Profiles



Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

**Unmet Needs** 

### **Key Questions**

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Epidermolysis bullosa drugs?

How many Epidermolysis bullosa drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Epidermolysis bullosa?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Epidermolysis bullosa therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Epidermolysis bullosa and their status?

What are the key designations that have been granted to the emerging drugs?

### **Key Players**

Abeona Therapeutics

Castle Creek Biosciences

**RHEACELL** 



**Aegle Therapeutics** Phoenix Tissue Repair, Inc. Lenus Therapeutics, LLC Amryt Pharma Wings Therapeutics Inc. Krystal Biotech, Inc. TWi Biotechnology, Inc. Berg Pharma Onconova Therapeutics, Inc. InMed Pharmaceuticals Inc. **Constant Therapeutics** Holostem Terapie Avanzate Kangstem Biotech **ProQR Therapeutics** Shionogi Ishin Pharmaceutical **Key Products** 

EB-101

FCX-007



allo-APZ2-EB
AGLE 102
PTR-01
RGN-137
Oleogel-S10
QR-313
beremagene geperpavec
AC-203
BPM31510
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S 005151
ISN 001



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Assessment by Stage and Molecule Type

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Comparative Analysis

EB-101: Abeona Therapeutics

**Product Description** 

Research and Development

**Product Development Activities** 

Drug profiles in the detailed report.....

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Comparative Analysis

PTR-01: Phoenix Tissue Repair

**Product Description** 

Research and Development

**Product Development Activities** 

Drug profiles in the detailed report.....

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Comparative Analysis

QR-313: ProQR Therapeutics

**Product Description** 

Research and Development



**Product Development Activities** 

Drug profiles in the detailed report.....

Preclinical and Discovery Stage Products

Comparative Analysis

TXA127: Constant Therapeutics

**Product Description** 

Research and Development

**Product Development Activities** 

Drug profiles in the detailed report.....

### **Inactive Products**

Comparative Analysis

Epidermolysis bullosa Key Companies

Epidermolysis bullosa Key Products

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