

Dystrophic Epidermolysis Bullosa - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Dystrophic Epidermolysis Bullosa - Pipeline Insight, 2021," report provides comprehensive insights about 20+ companies and 20+ pipeline drugs in Dystrophic 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

Dystrophic Epidermolysis Bullosa Understanding

Dystrophic Epidermolysis Bullosa: Overview

Dystrophic epidermolysis bullosa (DEB) is one of the major forms of epidermolysis bullosa. The signs and symptoms can vary widely among affected people. In mild cases, blistering may primarily affect the hands, feet, knees, and elbows. Severe cases often involve widespread blistering that can lead to vision loss, disfigurement, and other serious medical problems. DEB is caused by changes (mutations) in the COL7A1 gene and may be inherited in an autosomal dominant or autosomal recessive manner depending on the subtype. New blisters should be lanced, drained, and protected. Some patients need nutritional support, supplements, occupational therapy and/or

surgery depending on the associated features of the disease.

'Dystrophic 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 Dystrophic Epidermolysis Bullosa pipeline landscape is provided which includes the disease overview and Dystrophic Epidermolysis Bullosa treatment guidelines. The assessment part of the report embraces, in depth Dystrophic 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, Dystrophic 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 Dystrophic Epidermolysis Bullosa R&D. The therapies under development are focused on novel approaches to treat/improve Dystrophic Epidermolysis Bullosa.

Dystrophic Epidermolysis Bullosa Emerging Drugs Chapters

This segment of the Dystrophic 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.

Dystrophic Epidermolysis Bullosa Emerging Drugs

EB-101: Abeona Therapeutics

EB-101 is an autologous, gene-corrected cell therapy for 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. Currently, it is in Phase III stage of clinical trial evaluation to treat Dystrophic Epidermolysis Bullosa.

FCX-007: Castle Creek Biosciences

Castle Creek Biosciences' FCX-007 is an autologous dermal fibroblast genetically modified to express functional COL7 that is missing or deficient in these patients. Transduced with a lentiviral vector containing the COL7 producing gene, known as COL7A1, FCX-007 is injected directly into the papillary dermis of blisters and wounds where the protein enables formation of anchoring fibrils to hold the layers of skin together. FCX-007 offers the potential to address the underlying cause of RDEB by providing high levels of COL7 directly to the affected areas, thereby avoiding systemic treatment. Fibrocell is developing FCX-007 in collaboration with Precigen, Inc., a wholly owned subsidiary of Intrexon Corporation.

RGN-137: RegeneRx

RegeneRx has been developing RGN-137, a topical gel formulation of the peptide T α 4, as a novel treatment to accelerate dermal wound healing. RGN-137 was the first formulation of T α 4 to be used in human clinical trials based on research generated at the National Institutes of Health. RGN-137 represents a novel approach to dermal wound healing. Unlike growth factors, RGN-137 has been shown in preclinical studies to attenuate multiple healing pathways, such as apoptosis, angiogenesis, collagen deposition, and tissue inflammation. The gene for T α 4 has also been shown in preclinical studies to be one of the first to be upregulated when an injury occurs, suggesting that the peptide contributes to the healing process.

QR-313: ProQR Therapeutics

QR-313 is a potential first-in-class RNA-based oligonucleotide designed to address the underlying cause of dystrophic epidermolysis bullosa (DEB) due to mutations in exon 73 of the COL7A1 gene. Mutations in this exon can cause loss of functional collagen type VII (C7) protein. Absence of C7 results in the loss of anchoring fibrils that normally link the dermal and epidermal layers of the skin together. QR-313 is designed to exclude exon 73 from the mRNA (exon skipping) and produce a functional C7 protein, thereby restoring functionality of the anchoring fibrils. The clinical development of QR-313 is

supported with funding from EB Research Partnership and EB medical Research Foundation.

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

Dystrophic Epidermolysis Bullosa: Therapeutic Assessment

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

Major Players in Dystrophic Epidermolysis Bullosa

There are approx. 20+ key companies which are developing the therapies for Dystrophic Epidermolysis Bullosa. The companies which have their Dystrophic Epidermolysis Bullosa drug candidates in the most advanced stage, i.e. phase III include, Abeona Therapeutics.

Phases

DelveInsight's report covers around 20+ 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

Dystrophic 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

Intradermal

Intravenous

Oral

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Cell therapy

Gene therapies

Small molecules

Peptide

Protein

Small interfering RNA

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Dystrophic Epidermolysis Bullosa: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Dystrophic 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 Dystrophic Epidermolysis Bullosa drugs.

Dystrophic Epidermolysis Bullosa Report Insights

Dystrophic Epidermolysis Bullosa Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Dystrophic 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 Dystrophic Epidermolysis Bullosa drugs?

How many Dystrophic 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 Dystrophic Epidermolysis Bullosa?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Dystrophic 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 Dystrophic 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

Krystal Biotech

Amryt Pharma

RegeneRx

Rheacell

TWi Biotechnology

Shionogi & Co.

Quoin Pharmaceuticals

ProQR Therapeutics

Phoenix Tissue Repair

Aegle Therapeutics

Constant Therapeutics

Key Products

EB-101

FCX-007

B-VEC

Oleogel-S10

RGN-137

APZ 2

AC-203

Redasemtide

QRX004

QR-313

PTR-01

AGLE 102

TXA127

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