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Fanconi Anemia - Pipeline Review, H2 2020

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Abstracts

Fanconi Anemia - Pipeline Review, H2 2020

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Fanconi Anemia - Pipeline Review, H2 2020, provides an overview of the Fanconi Anemia (Hematological Disorders) pipeline landscape.

Fanconi anemia or FA, is a rare, inherited blood disorder that leads to bone marrow failure. The disorder also is called Fanconi’s anemia. FA is a type of aplastic anemia. In aplastic anemia, the bone marrow stops making or doesn't make enough of all three types of blood cells. Low levels of the three types of blood cells can harm many of the body's organs, tissues, and systems. Treatment is recommended for significant cytopenias, such as hemoglobin less than 8 g/dL, platelets fewer than 500/?L.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Fanconi Anemia - Pipeline Review, H2 2020, provides comprehensive information on the therapeutics under development for Fanconi Anemia (Hematological Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Fanconi Anemia (Hematological Disorders) pipeline guide also reviews of key players involved in therapeutic development for Fanconi Anemia and features dormant and discontinued projects. The guide covers therapeutics under Development by

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Companies/Universities/Institutes, the molecules developed by Companies in Phase II, Phase I, IND/CTA Filed, Preclinical and Discovery stages are 2, 1, 1, 2 and 2 respectively. Similarly, the Universities portfolio in Phase II and Preclinical stages comprises 3 and 2 molecules, respectively.

Fanconi Anemia (Hematological Disorders) pipeline guide helps in identifying and tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

Note: Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Fanconi Anemia (Hematological Disorders).

The pipeline guide reviews pipeline therapeutics for Fanconi Anemia (Hematological Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Fanconi Anemia (Hematological Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Fanconi Anemia (Hematological Disorders)

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therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Fanconi Anemia (Hematological Disorders)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Fanconi Anemia (Hematological Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it’s most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Fanconi Anemia (Hematological Disorders) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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Contents

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Featured News & Press Releases

May 12, 2020: Rocket Pharmaceuticals presents positive updates on RP-L102 program at the 23rd annual meeting of the American Society of Gene and Cell Therapy

Feb 24, 2020: Rocket Pharmaceuticals announces publication of manuscript evaluating mosaicism in Fanconi anemia

Dec 16, 2019: Rocket Pharmaceuticals receives the European Medicines Agency PRIME eligibility for RP-L102 gene therapy for Fanconi anemia

Dec 08, 2019: Rocket Pharmaceuticals presents promising preliminary results from phase 1 trial of commercial-grade RP-L102 “Process B” for Fanconi Anemia at 61st American Society of Hematology Annual Meeting

Dec 06, 2019: Rocket pharmaceuticals announces first patient treated in global registrational phase 2 study of RP-L102 “process B” for fanconi anemia

Nov 06, 2019: Rocket Pharmaceuticals to present preliminary phase 1 data of RP-L102 “Process B” for Fanconi Anemia at the 61st American Society of Hematology Annual Meeting

Oct 24, 2019: Rocket Pharmaceuticals presents first evidence of long-term improvement and stabilization in blood counts and durable mosaicism in RP-L102 “Process A” for Fanconi Anemia

Oct 15, 2019: Rocket Pharmaceuticals announces upcoming presentation on Gene Therapy 1 for Fanconi Anemia at the European Society of Gene and Cell Therapy Annual Congress

Sep 10, 2019: Rocket Pharmaceuticals announces publication of data from phase 1/2 trial of first-generation RP-L102 for Fanconi Anemia in Nature Medicine

Sep 04, 2019: Rocket Pharmaceuticals announces registration-enabling phase 2 plans for RP-L102 gene therapy for fanconi anemia following a supportive end-of-phase 1 FDA meeting

Aug 22, 2019: Rocket Pharmaceuticals announces clearance from the Spanish Agency for Medicines and Health Products for the phase 2 registration-enabling FANCOLEN-II study of RP-L102 for Fanconi Anemia

Apr 15, 2019: Rocket Pharmaceuticals announces upcoming presentations at the American Society of Gene and Cell Therapy 2019 Annual Meeting

Mar 12, 2019: Rocket Pharmaceuticals announces first patient dosed in phase 1 clinical trial of RP-L102 in the U.S.

Nov 27, 2018: Rocket Pharmaceuticals receives FDA Regenerative Medicine Advanced Therapy (RMAT) and Fast Track Designations for RP-L102 Gene Therapy for Fanconi Anemia

Nov 07, 2018: Rocket Pharmaceuticals announces FDA clearance of IND application for RP-L102 gene therapy for fanconi anemia

Appendix

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List Of Tables

LIST OF TABLES

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List Of Figures

LIST OF FIGURES

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