AND

« *LANDSCAPE IN…* »

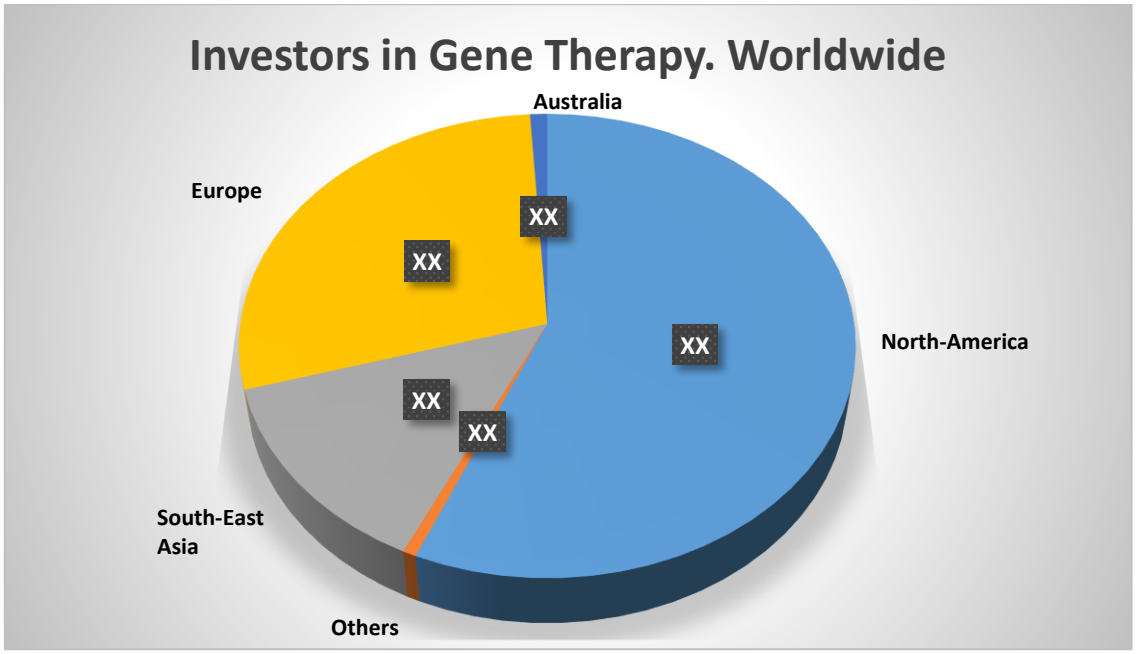
GENE THERAPY COMPANIES

UPDATED SEP 2019

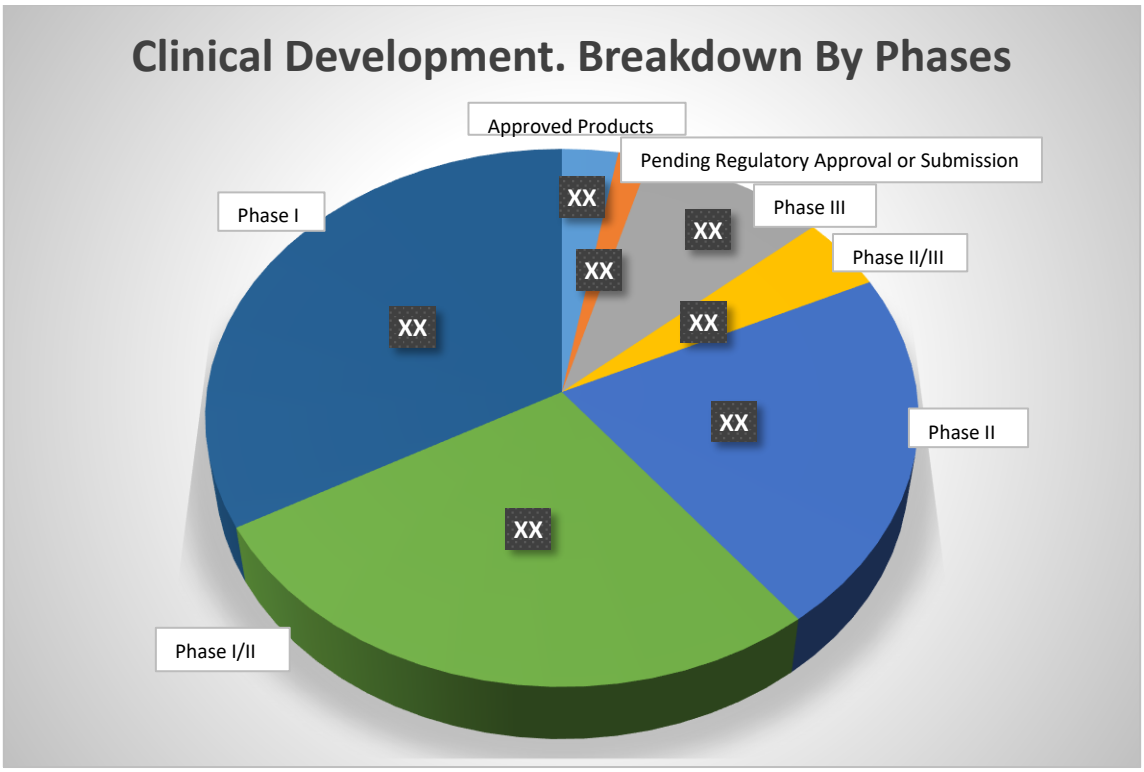
GLOBAL CONTENTS

EXAMPLES OF DIAGRAMS DETAILED IN THE FULL REPORT

INVESTORS IN GENE THERAPY COMPANIES



BREAKDOWN BY CLINICAL PHASES



BREAKDOWN BY CLINICAL PHASE (Extract)

Approved Products in the US and/or in the EU

- **Translarna® - PTC Therapeutics (USA)**

  (ataluren - inducer of ribosomal readthrough on nonsense mutation mRNA stop codons)

  Approved in August 2014 in the EU for the treatment of **Duchenne muscular dystrophy (DMD) resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older**

- **Strimvelis® - Orchard Therapeutics (GBR)**

  (autologous CD34+ cells transduced to express adenosine deaminase (ADA)

  Approved in May 2016 in the EU for the treatment of **adenosine deaminase severe combined immunodeficiency (ADA-SCID)**

And Several More….

You'll Have Access to the Complete Informations in the Full Report

Approval Pending/Filing To Be Submitted

- **Givosiran (ALN-AS1) - Alnylam Pharmaceuticals (USA**

  (siRNA directed against delta-aminolevulinic acid synthase 1 mRNA)
  NDA in the US and Application for a Market Authorization in the EUE filed in June 2019 for the treatment of **Acute Hepatic Porphyrias (AHP)**

- **AVXS-101 - Avexis (USA), a Novartis Company**

  (AAV9 vector containing SMN transgene)

  Filing expected in 2020 for the treatment of **Spinal Muscular Atrophy (SMA) type 2 and 3**

And Several More….

You'll Have Access to the Complete Informations in the Full Report

Product Candidates in Phase III studies

- **Acromegaly**

  - **ATL1103 sc Injection** (atesidorsen – antisense oligonucleotide designed to block growth hormone receptor (GHr) expression) - **Antisense Therapeutics (AUS)**

    Phase III completed - Early Access Program to be established in Europe

- **ADA-SCID**

  - **OTL-101** (LV-based gene therapy) - **Oxford BioMedica (GBR)**

And Several More….

You'll Have Access to the Complete Informations in the Full Report

DETAILED INFORMATIONS ON COMPANIES (Examples)
In-Depth Analysis of 234 Companies in the Full Report

Benitec (AUS)

General Informations

ASX: BLT**, NASDAQ:** BNTC

Year founded: 1995
Location: Suite 1201, 99 Mount Street, North Sydney NSW 2060, AUS
Phone: +61 (02) 9555 6986. Fax: +61 (02) 9818 2238. info@benitec.com

Website: https://benitec.com/, https://www.linkedin.com/company/benitecbiopharma-ltd/
FTE: 11-50

Management:

Jerel A Banks, M.D., Ph.D, Executive Chairman and CEO
(jbanks@benitec.com)
Oliver Kidd, Company Secretary
(okidd@benitec.com)
Michael Graham, PhD, Founding Scientist
(mgraham@benitec.com, https://au.linkedin.com/in/michael-graham-4874b743)
Greg Reyes, MD, PhD, Senior Scientific Advisor
(greyes@benitec.com)
Vanessa Strings-Ufombah, PhD, Senior Scientist
(vstrings-ufombah@benitec.com,https://www.linkedin.com/in/vanessa-strings-ufombah-phd/)
Megan Boston Head of Operations Australia
(mboston@benitec.com, https://www.linkedin.com/in/megan-boston-3b482b6b/)

Core Business in Gene Therapy: DNA-directed RNA Interference (ddRNAi)

Corporate Informations

Benitec is a biotechnology company developing a proprietary therapeutic technology platform that combines RNA interference with gene therapy for the goal of providing sustained, long-lasting silencing of disease-causing genes from a single administration. This proprietary platform is called **DNA-directed RNA interference (ddRNAi) and ddRNAi-based genetic medicines** developed by Benitec represent a pipeline of proprietary and partnered product candidates in several chronic and life-threatening human disease areas including:

■ **Oncology**: **BB-401 and BB-501** for the the treatment of Head & Neck Squamous Cell Carcinoma (HNSCC). Benitec acquired rights to BB-401 from Nant Capital.
■ **Rare disease**: **BB-301** for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD). In July 2018, Benitec has licensed to **Axovant Sciences** exclusive global rights for BB-301,

now named AXO-AAV-OPMD. Benitec and Axovant have also entered in a research collaboration for the development of five additional gene therapy products in neurological disorders. The first program for the treatment of two diseases - AXO-AAV-ALS intended for the treatment of amyotrophic lateral sclerosis (ALS), and AXO-AAV-FTD intended for the treatment for frontotemporal dementia (FTD) - were announced in July 2018, and the company plans to announce additional partnered programs in 2019.

■ **Retinal disease**: **BB-201** for the treatment of Wet Age Related Macular Degeneration (wet AMD)

■ **Infectious disease: BB-103** for the treatment of Hepatitis B Virus (HBV).

Benitec Biopharma is headquartered in Sydney, Australia. Its scientific operations are based in the San Francisco Bay Area.

Recent Fundings and Financial Highlights

-Market Capitalisation: 9.77 M$ (SEP 2019)

-Recent Fundings:

-JUN 2018: 6.2 MA$ (shares issued to Nant Capital)
-MAY 2018: 2.6 MA$ (private placement)
-MAR 2017: 5.4 MA$ (private placement)
-OCT 2016: 2.5 MA$ (private placement)
-AUG 2015: 18.8 MA$ (NASDAQ IPO)
-FEB 2014: 31.5 MA$ (private placement)

-For the Half-Year ended December 31, 2018

-Revenues: 16.32 MA$ (2017: 2.25 MA$)
-R&D expenses: 1.65 MA$ (2017: 3.29 MA$)
-G&A expenses: 5.61 MA$ (2017: 4.76 MA$)
-Net loss: 9.06 MA$ (2017: 5.8 MA$)
-Cash and cash equivalents: 23.18 MA$ (As of December 31, 2018)

-Benitec is listed on the Australian Securities Exchange (ASX) since 1997. It is also listed on NASDAQ since July 2015.

Pipeline

Five Products in HNSCC, OPMD, HBV and AMD

Indication	Product	Type / Mechanism of action	Discovery Preclinical	Development Phase			Approved Marketed
				Phase I	Phase II	Phase III	
HNSCC	BB-401	**Antisense.** DNA Plasmid that produces an antisense RNA that target the EGFR mRNA. Delivered intratumorally.	✓	✓	✓		
	BB-501	**ddRNAi** designed to silence the expression of EGFR	✓				

OPMD	BB-301	**ddRNAi. Gene therapy**. AAV vector expressing shRNA to knock down mutant PABP1 as well as a codon optimized, shRNA-insensitive, wildtype PABN1	✓	**Clinical program expected to begin in H2 2019**			
HBV	BB-103	**ddRNAi. Gene therapy**. AAV expressing anti-HBV shRNA modeled into miRNA backbones	**IND enabling**				
Wet AMD	BB-201	**ddRNAi. Gene therapy**. AAV with a recombinant DNA cassette, engineered to express steady state levels of three short hairpin RNA that inhibit VEGF-a, VEGF-b and PlGF	✓				

Clinical Trials

One Product for the Treatment of HNSCC

ID	Product	Phase and status	Start / Completion Date	Planned enrollment	Results / Comments	Indication	Collab. Nb of sites	Other ID	Last Update
NCT03433027	BB-401	II Active not recruiting	MAR 2018/ OCT 2019	16		HNSCC	6 Locations	BB-401-01	NOV 2018

Latest Developments related to Gene Therapies

Date	Subject / Title	Partner	Comments / Link
JUL 2019	Benitec announced a workforce reduction		Benitec's management has taken steps to streamline operations and ensure its primary ddRNAi product candidate, BB-301, will progress to receive meaningful data from clinical trials. In the third quarter 2019, Benitec conducted a workforce reduction of approximately 50% to align key staff members with the company's strategic goals. Press Release
JUN 2019	Update on Oculopharyngeal Muscular Dystrophy Program	Axovant (USA – CHE)	Benitec Biopharma announced the termination of the license and collaboration agreement with Axovant, as the Benitec team endeavors to conduct several additional exploratory analyses prior to the initiation of the clinical study in order to potentially improve the biological efficacy of the compound via further optimization of the proprietary delivery method employed to dose the target tissues. The termination of the License and Collaboration Agreement will be effective on September 3, 2019. Press Release
DEC 2018	Benitec Provides Update on BB-401 Cancer Treatment Program		Based on the initial analysis, the objective response rate required to support continued patient enrollment into the Phase II study was not achieved. Press Release

SEP 2018	Benitec Biopharma Announces the Appointment of Gregory R.Reyes, M.D., Ph.D. as Senior Scientific Advisor		Press Release
JUL 2018	Benitec Announces Global Licensing Agreement for BB-301 for Treatment of Oculopharyngeal Muscular Dystrophy and Broad Platform Collaboration with Axovant		Under the terms of the agreement, Benitec will receive an upfront cash payment of US$10 million and additional cash payments totaling US$17.5 million upon completion of four specific near-term manufacturing, regulatory and clinical milestones. Press Release
JUN 2018	Benitec announces Change of Company Secretary		Press Release

Latest Related Publications / Results

Reference	Authors, Location	Results / Comments	Link
***J Cachexia Sarcopenia Muscle.* 2019 May 7. doi:10.1002/jcsm.12438**	Harish P et al. Centres of Gene and Cell Therapy and Biomedical Sciences, School of Biological Sciences, Royal Holloway-University of London, Surrey, UK.	The study supports the clinical translation of antibody-mediated inhibition of myostatin as a treatment of oculopharyngeal muscular dystrophy. This strategy has implications to be used as adjuvant therapies with gene therapy based approaches, or to stabilize the muscle prior to myoblast transplantation.	Abstract Full Text
***Oral Maxillofac Surg Clin North Am.* 2019 Feb; 31(1):117-124.**	Farmer ZL et al. Levine Cancer Institute, 1021 Morehead Medical Drive, Charlotte, NC 28204, USA.	This article provides insight into some gene therapy targets and varied techniques being evaluated for patients with head and neck cancer. Techniques include corrective gene therapy, cytoreductive gene therapy, and gene editing, in addition to a discussion on gene therapy vectors.	Abstract
***Methods Mol Biol*. 2019;1974:393-408.**	Xu L, Yang H, Section of Nephrology, Department of Internal Medicine, Yale University School of Medicine, New Haven, CT, USA.	This article describes the design and synthesis of fluorescently labeled, folic acid-decorated polyamidoamine (PAMAM) generation 4 (G4) dendrimer conjugates for HNSCC-targeted gene delivery.	Abstract
***Oncol Lett.* 2019 Feb; 17(2):1953-1961.**	Wang C et al. The First Affiliated Hospital of Anhui Medical University, Hefei, Anhui 230022, P.R. China.	Exosome-delivered TRPP2 siRNA inhibits the epithelial-mesenchymal transition of FaDu cells.	Abstract Full Text
***Nat Commun.* 2017 Mar 31;8:14848.**	Malerba A et al. School of Biological Sciences, Royal Holloway, University of London, Egham Hill, Egham, TW20 0EX Surrey, UK.	PABPN1 gene therapy for oculopharyngeal muscular dystrophy.	Abstract Full Text

Akcea Therapeutics (USA)

General Informations

NASDAQ: AKCA

Year founded: 2014
Location: 22 Boston Wharf Road, 9th Floor, Boston MA 02210, USA
Phone: +1 (617) 207-0202. info@akceatx.com

Website: XXXX
FTE: XX

Management:

Sarah Boyce, President (XXXX@akceatx.com)
Paula Soteropoulos, CEO (XXXX@akceatx.com)
XXX, COO (XXXX@akceatx.com)
XXX, CMO (XXXX@akceatx.com)
XXX, Chief Development Officer (XXXX@akceatx.com)
XXX, VP Global Head of TTR Strategy (XXXX@akceatx.com)
XXX VP Global Head and General Manager (XXXX@akceatx.com)
XXX, Head of Europe (XXXX@akceatx.com)
XXX, VP Market Access (XXXX@akceatx.com)
XXX, VP Medical Affairs (XXXX@akceatx.com)
XXX, VP Pharmacovigilance and Drug Safety (XXXX@akceatx.com)

Core Business in Gene Therapy: Antisense-derived Drugs for hATTR and Rare Cardiometabolic Lipid Disorders

Corporate Informations

Akcea Therapeutics, an affiliate of Ionis Pharmaceuticals, is a commercial stage biopharmaceutical company focused on developing and marketing drugs to treat patients with rare and serious diseases. The company has now **two approved products**:

- **XXXX**……..
- **XXXX**……..

Akcea Therapeutics is building its commercial infrastructure to support these two drugs and other products of its pipeline.The company has launched Akcea Connect™, a drug treatment program made up of dedicated, regionally-based nurse case managers in the United States……
……...
……...

As an affiliate of Ionis Pharmaceuticals, the company has a robust portfolio of development-, registration- and commercial-stage drugs covering multiple targets and diseases using antisense therapeutics:

- **XXXX**……..
- **XXXX**……..
- **XXXX**……..
- **XXXX**……..

Recent Fundings and Financial Highlights

-Market Capitalisation: XX BUS$ (SEP 2019)
-Valuation at IPO: 513.5 MUS$ (JUL 2017)
-Akcea Therapeutics went public on JUL 2017 (125 MUS$ raised)

-For the Year ended December 31, 2018

-Net revenue 2018: XX MUS$
-R&D expenses 2018: XX MUS
-G&A expenses 2018: XX MUS$
-Net loss 2018: XX MUS$
-Cash, cash equivalents ad short term investments: XXX (As of December 31, 2018)

Pipeline

Six Products for Seven Indications

Indication	Product	Type / Mechanism of action	Discovery/ Preclinical	Development Phases			Approved / Marketed
				Phase I	Phase II	Phase III	
Hereditary transthyretin-mediated amyloidosis (hATTR)	Tegsedi® (inotersen)	**Antisense** oligonucleotide designed to reduce the production of transthyretin	✓	✓	✓	✓	**Approved in Canada, EU and US**
XXXXX	XXXXX	**Antisense** oligonucleotide targeted to XXXX	✓	✓	✓	✓	**XXXXXX**
XXXXX			✓	✓	✓	✓	
XXXXX	XXXXX	**Antisense** oligonucleotide that inhibits the production of XXXXX	✓	✓	✓	**In preparation**	
XXXXX	XXXXX	**Antisense** oligonucleotide that inhibits XXXXX	✓	✓	**Data to be reported in 2020**		
XXXXX	XXXXX	**Antisense** oligonucleotide that inhibits XXXXX	✓	✓	✓		
XXXXX			✓	**I-II Data to be reported in 2020**			

XXXXX	XXXXX	**Antisense** oligonucleotide designed to inhibit the XXXXX	✓	**I-II**		**Initiation planned in 2019**	

Orphan Drug Designations

Product	Indication	EMA	FDA
XXXXX	XXXXX	JUL 2016	
XXXXX	XXXXX		JUL 2015

Clinical Trials

Seven On-going Clinical Trials In Multiple Lipid Disorders

ID	Product	Phase Status	Start / Completion Date	Planned enrollment	Results / Comments	Indication	Collab. nb of sites	Other ID	Last Update
XXXXX	XXXXX	III Active, not recruiting	DEC 2015/ OCT 2020	69	NI	XXXXX	XXXXX	XXXX	NOV 2018
XXXXX		II/III Active, not recruiting	OCT 2015/ SEP 2021	60	NI	XXXXX	XXXXX	XXXX	AUG 2018
XXXXX	XXXXX	EAP Available	-	-	XXXXX	XXXXX	CaligorRx	XXXX	JUN 2018
XXXXX	XXXXX	II Recruiting	DEC 2017/ SEP 2018	3	NI	XXXXX	XXXXX	XXXX	AUG 2018
XXXXX		II Recruiting	DEC 2017/ MAY 2019	144	NI	XXXXX	XXXXX	XXXX	SEP 2018
XXXXX		II Active, not recruiting	MAY 2018/ AUG 2019	3	NI	XXXXX	XXXXX	XXXX	JAN 2019
XXXXX	XXXXX	II Recruiting	JAN 2018/ MAY 2019	100	NI	XXXXX	XXXXX	XXXX	DEC 2018
XXXXX	XXXXX	I Recruiting	MAR 2018/ JAN 2019	16	NI	XXXXX	XXXXX	XXXX	OCT 2018

Latest Developments

Date	Subject / Title	Partner	Comments / Link
XXX 2019	hATTR ……. Hereditary ATTR Amyloidosis….	Ambry Genetics (USA)	Press Release
XXX 2019	New Long-Term Data…… as an Oral Presentation at 2019 American Academy of Neurology Annual Meeting (AAN)		Press Release
XXX 2019	Akcea and Ionis …. in the European Union	Ionis Pharmaceuticals (USA)	Press Release
XXX 2019	AKCEA-XXXXX….. .. Option to License	Novartis (CHE)	Novartis….. Press Release
XXX 2018	Phase 2 Results on AKCEA-XXX Presented in ……..	Novartis (CHE)	Press Release
XXX 2018	Akcea Announces Changes ……		….. replaced … on Akcea Board of Directors Press Release
XXX 2018	Akcea Announces Its Access ….		Press Release
XXX 2018	Akcea and Ionis Receive FDA….	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea and Ionis Announce Approval of ….	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea and Ionis Report ….	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea and Ionis Receive ….from FDA	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea Therapeutics and PTC Therapeutics .	PTC Therapeutics (USA)	Press Release
XXX 2018	Akcea Announces Publication of ….		Press Release
XXX 2018	Akcea and Ionis Announce Approval of….	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea Announces Completion …		Press Release
XXX 2018	Akcea and Ambry Genetics to Launch …..	Ambry Genetics (USA)	Press Release

XXX 2018	Akcea Therapeutics Appoints …		Press Release
XXX 2018	FDA Advisory Committee Votes …..		Press Release
XXX 2018	Akcea Announces Expansion of ….		Press Release
XXX 2018	Akcea and Ionis Complete Licensing ….	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Sarah Boyce Joins Akcea Therapeutics as President and Member of the Board of Directors		Press Release
XXX 2018	Akcea Convenes First FCS Global Connection Summit		Press Release
XXX 2018	MHRA Grants …		Press Release
XXX 2018	Ionis and Akcea Partner to …..	Ionis Pharmaceuticals (USA)	Press Release
XXX 2018	Akcea Therapeutics Canada Announces the Launch ..		Press Release
XXX 2018	Akcea Completes …		Press Release
XXX 2018	Akcea Initiates …		Press Release

Latest Related Publications / Results

Reference	Authors, Location	Results / Comments	Link
***Pharmaceuticals* (Basel). 2019 XXX; 12(2).**	XXXXXXXX…… Portugal	XXXX: An Antisense Oligonucleotide Approved ….	Abstract Full Text
***Curr…..* 2019 XXX; 21(8):30.**	XXXXXXXX …. Denmark.	Antisense …..	Abstract
***Muscle Nerve.* 2019 XXX. doi: XXX**	XXXXXXXX …. USA.	….. Hereditary Transthyretin Amyloidosis …….	Abstract
***Neurodegener XXXX.* 2019 XXX; X(1):XX-XX**	XXXXXXXX …. USA.	Inotersen …….	Abstract Full Text
***J Manag XXXX.* 2019 XXX; 25(X): XX-XX**	XXXXXXXX …. USA.	TTR Gene …….	Full Text

Mult Scler XXXX. **2019 XXX; 25(X): XX-XX**	XXXXXXXX …. USA.	Multiple Sclerosis…….	Abstract
***Am* XXXX ….** **2018 XXX, 1X(X): XX-XX**	XXXXXXXX …. USA.	Treatment ……..	Abstract Full Text
J Clin XXXX. **2018 XXX; XX(X):XXXX- XXXX.**	XXXXXXXX …. South Africa.	Characterizing familial ….: Baseline data of ……	Abstract Full Text
Drugs. **2018 XXX; XX(XX):XXXX- XXXX.**	XXXXXXXX …. NZ	This article summarizes the milestones in ….	Abstract
N Engl J Med **2018; XXX:XX-XX**	XXXXXXXX …. USA.	This article develops results from pivotal study of….	Full Text

Iveric Bio (USA)

General Informations

NASDAQ: ISEE

Year founded: 2007
Location: One Penn Plaza Suite 3520, New York, NY 10119, USA
Phone: + 1 212.845.8200
700 Alexander Park Suite 302, Princeton, NJ 08540, USA
Phone: +1 609.945.6050. info@Ivericbio.com

Website: XXXX
FTE: XX

Management:
Glenn P. Sblendorio, President & CEO (XXX@Ivericbio.com)
XXX, MD, CMO (XXX@Ivericbio.com)
XXX, COO (XXX@Ivericbio.com)
XXX, Chief Clinical Operations Officer (XXX@Ivericbio.com)
XXX, Head of CMC Gene Therapy (XXX@Ivericbio.com)
XXX, Director of Research (XXX@Ivericbio.com)
XXX, CBO (XXX@Ivericbio.com)
XXX VP, General Counsel & Corporate Secretary (XXX@Ivericbio.com)
XXX, VP Investor Relations & Corporate Communications (XXX@Ivericbio.com)

Core Business in Gene Therapy: AAV-based Gene Therapy, « MiniGene » Therapy for Orphan and Age-Related Retinal Diseases

Corporate Informations

Iveric Bio was previously known as **Ophthotech**. The company announced in April 2019, that as part of its transition strategy to focus on……
……..
…….....

In XXX 2018, the company has established…….
……..……
……..……..
.....

In XXX 2018, the company has acquired **exclusive development and commercialisation rights** to an AAV gene therapy product for the treatment of XXXX through a license agreement with the **XXXXXX**. In addition to this agreement……
……..……
……..……..
.....

In XXX 2018, the company has then entered into an **exclusive option agreement** with……
……
……
.....

Recent Fundings and Financial Highlights

-Market Capitalisation: Approx. XX MUS$ (SEP 2019)
-Main shareholders: XXXX (USA), XXXX (USA),XXXX (USA), XXXX (USA), XXXX (USA)

-For the Year ended December 31, 2018

-Revenues: XX MUS$ (2017: XX MUS$)
-R&D expenses: XX MUS$ (2017: XX MUS$)
-G&A expenses: XX MUS$ (2017: XX MUS$)
-Net loss: XX MUS$ (2017: XX MUS$)
-Cash and cash equivalents: XX MUS$ (As of December 31, 2018) - Cash runway through end of XXXX.

Pipeline

Seven Programs in Inherited Retinal Diseases

Indication	Product	Type / Mechanism of action	Discovery Preclinical	Development Phase			Approved / Marketed
				Phase I	Phase II	Phase III	
XXXXX	XXXXX	**Pegylated RNA aptamer**	✓	✓	**IIb Initial data expected in 4Q 2019**		
XXXXX			✓	✓	**IIb Initial data expected in 2H 2020**		
XXXXX	XXXXX	**AAV Gene therapy.** Knockdown and ….	✓	**I/II Initiation planned in 2H 2020**			
XXXXX	XXXXX	**AAV Gene therapy.** AAV2 vector designed to deliver ….	✓	**I/II Initiation planned in 1H 2021**			
XXXXX	XXXXX	**AAV minigene therapy.** Replacement of the mutated gene …..	✓				
XXXXX	XXXXX	**AAV minigene therapy.**	✓				

		Replacement of the mutated gene ….					
XXXXX	XXXXX	**AAV minigene therapy.** Replacement of the mutated gene with …	✓				
XXXXX	XXXXX	**AAV Gene therapy.**	✓				

Clinical Trials

One Product in XXXXX and XXXXX

ID	Product	Phase and status	Start / Completion Date	Planned enrollment	Results / Comments	Indication	Collab. Nb of sites	Other ID	Last Update
NCT03364153	XXXXX	IIb Active, not recruiting	JAN 2018/ SEP 2020	95	Initial data expected in 2H 2020	XXXXX	39 Locations	XXXX	MAR 2019
NCT02686658		IIb Active, not recruiting	JAN 2016/ NOV 2019	200	XXXXX	XXXXX	78 Locations	XXXX	NOV 2018

Latest Developments related to Gene Therapies

Date	Subject / Title	Partner	Comments / Link
XXX 2019	Iveric bio Announces Successful ….	UMMS (USA)	Iveric Bio has exercised its option and entered into an exclusive global license agreement……. Press Release
XXX 2019	Iveric bio Enters into Strategic …	Paragon, a unit of Catalent Biologics (USA)	Iveric Bio has engaged Paragon… Press Release
XXX 2019	Ophthotech Transitions to a Gene Therapy …		Press Release
XXX 2019	Ophthotech Obtains Exclusive ….	University of... (USA)	Press Release
XXX 2019	Ophthotech Announces the Addition of Ophthalmic Industry Leader,..		Press Release
XXX 2018	Ophthotech Announces the Addition of Ophthalmic Industry Leader,…		Press Release
XXX 2018	Ophthotech Announces Results from Phase 2a Safety Trial of …		The combination therapy was …. Press Release

Latest Related Publications / Results

Reference	Authors, Location	Results / Comments	Link
***Expert XXXX*. 2019 XXX; XX(X):XXX-XXX.**	XXXXX et al…… USA.	Complement inhibition as ……	Abstract
***Expert XXXX*. 2018 XXX; XX(X):XXXX-XXXX.**	XXXXX et al…… USA.	ABCA4 gene therapy……	Abstract
***PNAS* 2018 XX 4; XXX(XX):EXXXX-EXXXX.**	XXXXX et al…… USA.	Mutation-independent rhodopsin gene therapy…..	Abstract Full Text
***PNAS* 2018 XXX; XXX(XX):EXXXX-EXXXX**	XXXXX et al…… USA.	BEST1 gene therapy ……	Abstract Full Text
***Hum Gene Ther.* 2018 Jan; 29(1):42-50.**	XXXXX et al…… USA.	Gene Therapy Using a miniCEP290 Fragment …..	Abstract Full Text

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24, avenue du Prado
13006 Marseille. France
Phone: 33 (0)668 071 907
Contact: olivier@octopusyx.fr