

# Value of Gene Therapy & Significance

David Barrett  
ASGCT Executive Director

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Advancing knowledge, awareness,  
and education of gene and cell therapy



# Thank you!

- Adora Ndu & Maritza McIntyre
- Francesca Cook & Max Bronstein
- Tim Hunt & Keith Joung
  
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SAREPTA  
THERAPEUTICS

# Keeping Current with the Pipeline

## Events and Publications

- ASGCT
- Molecular Therapy
- MIT NEWDIGS
- Duke Margolis Center

Molecular Therapy  
Review

### The Landscape of Early Clinical Gene Therapies outside of Oncology

Laure Rittié,<sup>1</sup> Takis Athanopoulos,<sup>2</sup> Miguel Calero-Garcia,<sup>2</sup> Marie L. Davies,<sup>2</sup> David J. Dow,<sup>2</sup> Steven J. Howe,<sup>3</sup> Alastair Morrison,<sup>4</sup> Ida Ricciardelli,<sup>2</sup> Aurore Saudemont,<sup>2</sup> Laurent Jespers,<sup>2</sup> and Timothy M. Clay<sup>1</sup>

<sup>1</sup>GSK R&D Cell and Gene Therapy Discovery Research, UP1410, 1250 S. Collegeville Road, Collegeville, PA 19426, USA; <sup>2</sup>GSK R&D Cell and Gene Therapy Discovery Research, 6F, Gunnels Wood Road, Stevenage, Herts SG1 2NY, UK; <sup>3</sup>GSK R&D Cell and Gene Therapy Process Research, 6F, Gunnels Wood Road, Stevenage, Herts SG1 2NY, UK; <sup>4</sup>GSK R&D Worldwide Business Development, 5G104, Gunnels Wood Road, Stevenage, Herts SG1 2NY, UK



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journal homepage: [www.elsevier.com/locate/jval](http://www.elsevier.com/locate/jval)

Themed Section: Curative Therapies

### Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare System

Casey Quinn, PhD,\* Colin Young, PhD, Jonathan Thomas, BSc, Mark Trusheim, MSc, and the MIT NEWDIGS FoCUS Writing Group  
Center for Biomedical Innovation, Massachusetts Institute of Technology, Cambridge, Massachusetts, USA.



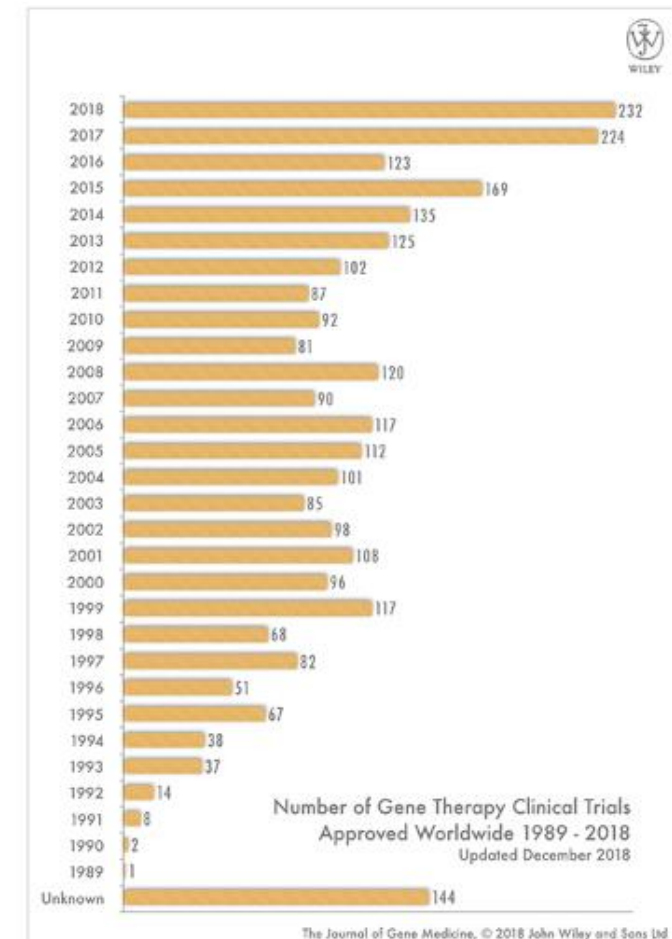
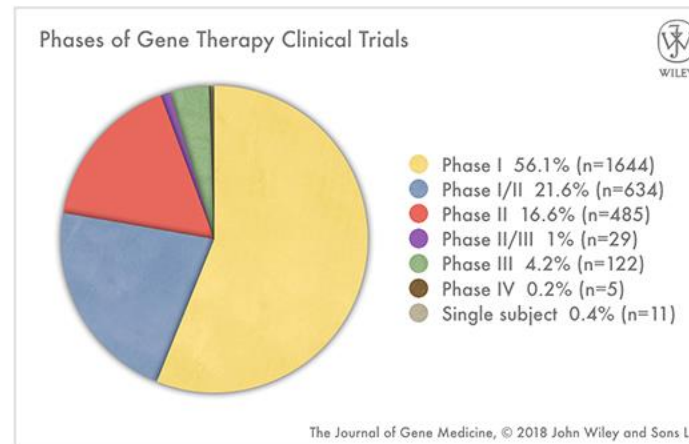
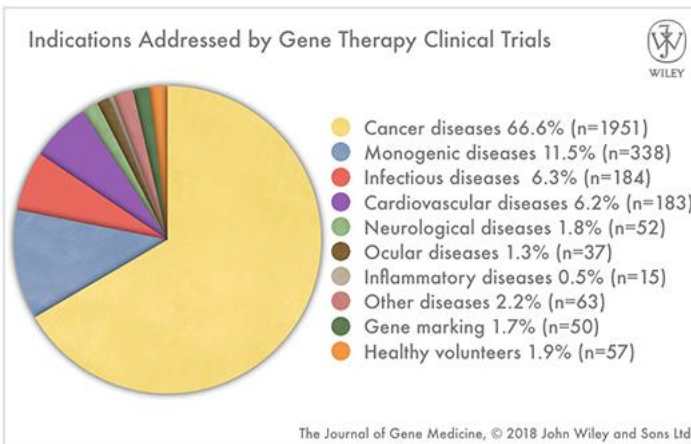
# Keeping Current with the Pipeline

- Clinicaltrials.gov

The screenshot displays the ClinicalTrials.gov website. At the top, the NIH logo and "U.S. National Library of Medicine" are visible, along with the site name "ClinicalTrials.gov" and a navigation menu with links for "Find Studies", "About Studies", "Submit Studies", "Resources", and "About Site". A blue banner below the header states: "ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world." The main content area is divided into two columns. The left column features the text: "Explore 320,846 research studies in all 50 states and in 209 countries." followed by a brief description of the site and an "IMPORTANT" notice regarding the evaluation of listed studies. The right column contains a "Find a study" search form with fields for "Status" (radio buttons for "Recruiting and not yet recruiting studies" and "All studies"), "Condition or disease" (with a placeholder example "breast cancer"), "Other terms" (with a placeholder example "NCT number, drug name, investigator name"), and "Country". A "Search" button and a link to "Advanced Search" are located below the form. At the bottom of the page, there are three navigation links: "Patients and Families", "Researchers", and "Study Record Managers".

# Keeping Current with the Pipeline

- Gene Therapy Clinical Trials Worldwide



# Keeping Current with the Pipeline

- ARM Anticipated Clinical Data & Events

**REGENXBIO**  
RGX-314



Ophthalmology


**Modality**  
Gene delivery

**Indication**  
Diabetic retinopathy

**Trial Milestone**  
IND to be submitted in 2H 2019

**Clinical Stage: Ph 2B**

**Audentes Therapeutics**  
AT132



Rare Disease  
Endocrine, metabolic, and genetic

**Modality**  
Gene delivery

**Indication**  
X-linked myotubular myopathy (XLMM)

**Submission / Approval**  
Expects to submit BLA in US in 2H 2020

**Clinical Stage: Ph 1/2**

*For more information: NCT03199469*

Alliance for Regenerative Medicine

## Anticipated Clinical Data & Events

To begin, select from the form options on the right. View the information online, or download in a CSV file to import into your spreadsheet app.

[SUBMIT A MILESTONE](#)

### Record Search

KEYWORD SEARCH:

MODALITY  
-- Select one --

INDICATION AREA  
-- Select one --

DISEASE RARITY:  
Show All

EVENT TYPE  
-- Select one --

SORT RESULTS BY  
Company

[SUBMIT](#)

Search Results

191 Records Found

[Download CSV](#)

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# Keeping Current with the Pipeline

## **ASGCT Clinical Trials Finder**

# ASGCT Clinical Trial Finder

- Driven by clinicaltrials.gov
- Curated for gene and cell therapy trials only
- US-based
- Updated in real time

The screenshot shows the ASGCT Clinical Trials Finder website. At the top, there is a navigation bar with links for ANNUAL MEETING, EDUCATION, RESEARCH, ADVOCACY, MEMBERSHIP, EVENTS, and ABOUT. The main heading reads "Welcome to the American Society of Gene & Cell Therapy's Clinical Trials Finder!" followed by a brief description of the search tool. Below this, there is a "View Trials (429)" section with a "Hide Filter Options" button. The filter section includes dropdown menus for "Therapeutic Area" (set to "All Therapeutic Areas") and "Diagnosis" (set to "All Diagnoses"), with a "Clear All Filters" link. Below these are input fields for "Modality" (Select Modalities), "Phase" (Select Phases), "Location" (Type zip code or city), "Drug" (Type to Select Drugs), "NCT ID" (Type to select NCT ID), and "Title" (Enter Title). A "location filter off" slider is also present. At the bottom, a table displays search results with columns for Title, Phase, Modalities, Interventions, and Location.

Title	Phase	Modalities	Interventions	Location
Efficacy Study on the Strategy of HSV-Tk Engineering Donor Lymphocytes to Trea...	3	Stem Cell Therapy	cyclophosphamide, HSV-Tk, T-cell...	View Locations ▾
Stem Cell Transplantation From HLA Partially-Matched Related Donors for...	3	Chemotherapy, Radiation Therapy,...	CD34+ haploidentical stem cells,...	USA - NY - New Hyde Park
A Phase III Trial Evaluating Chemotherapy and Immunotherapy fo...	3	Chemotherapy, Immunotherapy,...	Epstein Barr virus (EBV) specific...	View Locations ▾
Safety and Efficacy of ATIRI01 as		Chemotherapy	allogeneic T cell	



# Searchable by

- Therapeutic Area
- Modality
- Phase
- Title Keyword

Welcome to the American Society of Gene & Cell Therapy's Clinical Trials Finder!

Search hundreds of active and recruiting clinical trials in gene and cell therapy across the United States by selecting a therapeutic area or diagnosis to narrow the results—from there, focus your results by adding additional parameters from the selections below. All data is pulled from ClinicalTrials.gov and updated daily to ensure accuracy.

**View Trials (429)** Hide Filter Options

Therapeutic Area: All Therapeutic Areas | Diagnosis: All Diagnoses Clear All Filters

Modality: Select Modalities | Phase: Select Phases | Location: Type zip code or city

Drug: Type to Select Drugs | NCT ID: Type to select NCT ID

Title: Enter Title

Title	Phase	Modalities	Interventions	Location
Efficacy Study on the Strategy of HSV-Tk Engineering Donor Lymphocytes to Trea...	3	Stem Cell Therapy	cyclophosphamide, HSV-Tk, T-cell...	View Locations ▾
Stem Cell Transplantation From HLA Partially-Matched Related Donors for...	3	Chemotherapy, Radiation Therapy,...	CD34+ haploidentical stem cells,...	USA - NY - New Hyde Park
A Phase III Trial Evaluating Chemotherapy and Immunotherapy fo...	3	Chemotherapy, Immunotherapy,...	Epstein Barr virus (EBV) specific...	View Locations ▾
Safety and Efficacy of ATIRI01 as		Chemotherapy,	allogeneic T cell	

AMERICAN SOCIETY of  
**GENE & CELL  
THERAPY**

ANNUAL MEETING | EDUCATION | RESEARCH | ADVOCACY | MEMBERSHIP | EVENTS | ABOUT

## Gene Transfer Clinical Study in X-Linked Myotubular Myopathy

← Back

Phase: 1/2

Interventions: adeno-associated virus vector containing human MTM1 gene

Modalities: Gene Therapy, Viral Vector

NCT03199469

Lead Sponsor: Audentes Therapeutics

Full Title: ASPIRO: A Phase 1/2, Randomized, Open-Label, Ascending-Dose, Delayed-Treatment Concurrent Control Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT132, an AAV8-Delivered Gene Therapy in X-Linked Myotubular Myopathy (XLMTM) Patients

Diagnosis: X-Linked Myotubular Myopathy

Study Information

Brief Summary | **Detailed Description** | Study Design | Eligibility | Outcome Measures

# Features

- Simplified layout
- All pertinent information on the trial, driven by CT.
- Designed for patients and professionals



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**GENE & CELL  
THERAPY**

**Advancing knowledge, awareness,  
and education of gene and cell therapy**

