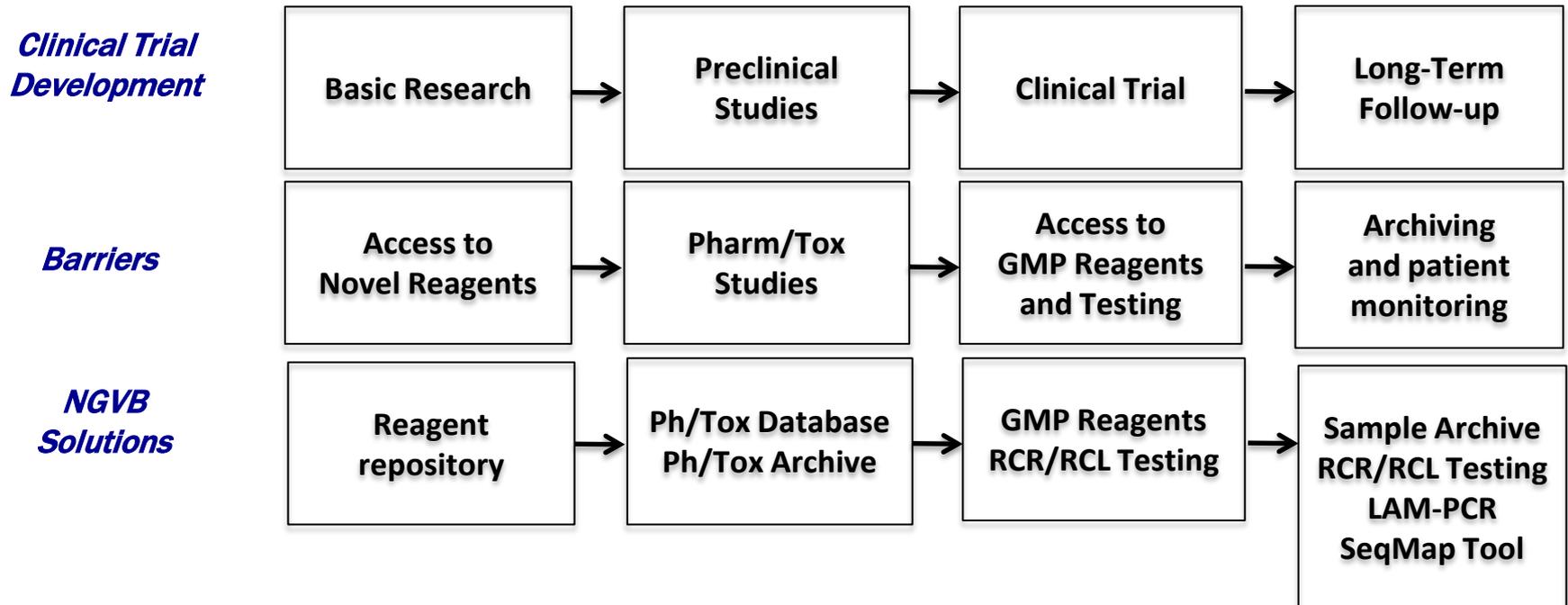


NATIONAL GENE VECTOR BIOREPOSITORY

A NHLBI FUNDED GENE THERAPY RESOURCE

Spectrum of NGVB Services

National Gene Vector Biorepository



Policy and Procedure Manual

National Gene Vector Biorepository

- Non-for-profit eligible to receive NIH funding
- Follow regulatory guidelines
- acknowledge the NGVB on all relevant publications
- agree to indemnification of the NGVB and NIH



Information

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- [Policies and Procedures](#)
- [What's New](#)
- [Contact Us](#)

Archiving Services

Insertion Site Analysis

Pharm/Tox Resources

Reagent Repository

Educational Resources

My NGVB

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user name:

password:

[Get user name and password](#)
[Can't remember your password?](#)

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Welcome to the NGVB

The **National Gene Vector Biorepository** (NGVB) is a resource for academic investigators and others conducting gene therapy research. The NGVB Coordinating Center (www.ngvbcc.org) is located in the Department of Medical and Molecular Genetics at the Indiana University School of Medicine. It is made possible through a grant from the National Center for Research Resources, National Institutes of Health.

Efforts in the NGVB are focused in four areas: Archiving Services; Insertional Site Analysis, Pharmacology and Toxicology Resources, and a Reagent Repository.

The databases and educational resources are open to everyone. Other services are limited to gene therapy investigators working in academic or other non-profit organizations. Please click on [About the NGVB](#) to see if you are eligible. Click on the boxes on your right to find out more about the available resources.

[About/Contact/Search](#)

Archiving Services

Click [here](#) to learn about services for clinical, toxicology, and other samples.

Insertion Site Analysis

Click [here](#) to learn about the SeqMap bioinformatics tool and get help with vector insertion analysis.

Pharm/Tox Resources

Click [here](#) to visit the NGVB Pharm/Tox database or the NGVB Pharm/Tox Tutorial.

Reagent Repository

Click [here](#) to visit the NGVB Reagent Repository.

The NGVB is supported by the NIH's [National Center for Research Resources](#) through a grant to the [Indiana University School of Medicine Department of Medical and Molecular Genetics](#)

Reagent Repository

National Gene Vector Biorepository

- ▣ AAV vector plasmids (courtesy of Jude Samulski)
- ▣ Adenoviral vector stocks (Lowenstein/Castro)
- ▣ Retroviral vectors
- ▣ Lentiviral packaging line (J. Gray)
- ▣ Certified Cell Lines
 - HEK293
 - HEK293T
 - Phoenix cell lines
 - PG13
 - G7b-1

Reagent Repository

All	Reagent Type ▼	Vector Classification ▼
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To order reagents: Select the reagents you want to see from the pull-down menu above and add them to your cart.

Examples:

- Select **All** to see all reagents
- Select **Reagent Type -> Cell Line -> AAV** to see all AAV cell lines
- Select **Reagent Type -> Cell Line** to see all cell lines

To continue to shop for reagents hit the reagents button at the top. To checkout hit the cart button to finalize your order.

Reagent Repository

All	Reagent Type ▼	Vector Classification ▼
	Antibody ▶	All
	Cell Line ▶	AAV
	Plasmid ▶	Adenovirus
	Vector ▶	HSV
		Lentivirus
		Retrovirus

To order
pull-down

Example

- Select **Reagent Type**
- Select **Reagent Type**
- Select **Reagent Type**

to see from the
cart.

AV to see all AAV

to see all cell lines

To continue to shop for reagents hit the reagents button at the top. To checkout hit the cart button to finalize your order.

Reagents

Reagents with type **Cell Line** and vector classification **Lentivirus**

Name	Type	Vector Classifications	Description	In Cart
C8166 cells	Cell Line	Lentivirus	This T cell line is available from the NIH AIDS repository and investigators should see material from this source for most applications (https://www.aidsreagent.org). If investigators are looking to compare RCL assay sensitivity and are collaborating with the IU Vector Production Facility, this cell line may be obtained from the NGVB to allow direct comparison.	
GPRG	Cell Line	Lentivirus	GPRG is a 293T based packaging cell line for the production of lentiviral vector particles with a VSV-G pseudotype, as described in Throm, et al. The cell line expresses HIV gagpol polyprotein constitutively, and HIV Rev and VSV-G under the Tet-off system.	
HEK293T	Cell Line	Lentivirus Retrovirus	HEK293T cells are a human cell line that can be transfected with plasmid DNA at high efficiency. The cell line was derived from the HEK293 cell line by the addition of the SV40 large T antigen that has been shown to increase vector production of some viral vectors. It is commonly used for production of retroviral, lentiviral and other viral vectors using the transient transfection method.	
HEK293T/CD4 cells	Cell Line	Lentivirus Retrovirus	HEK293T/CD4 is a human cell line that can be transfected with plasmid DNA at high efficiency. The cell line was derived from the HEK293 cell line by the addition of the SV40 large T antigen that has been shown to increase vector production of some viral vectors. It is commonly used for production of retroviral, lentiviral and other viral vectors using the transient transfection method. The cell line has been modified to express the CD4	
K562/Lentiviral Integration Clone 6	Cell Line	Lentivirus	K562 with lentiviral integration Clone 6 K562cells (human immortalised myelogenous leukaemia line) were transduced with lentiviral vector to produce clones stably expressing	

Reagent

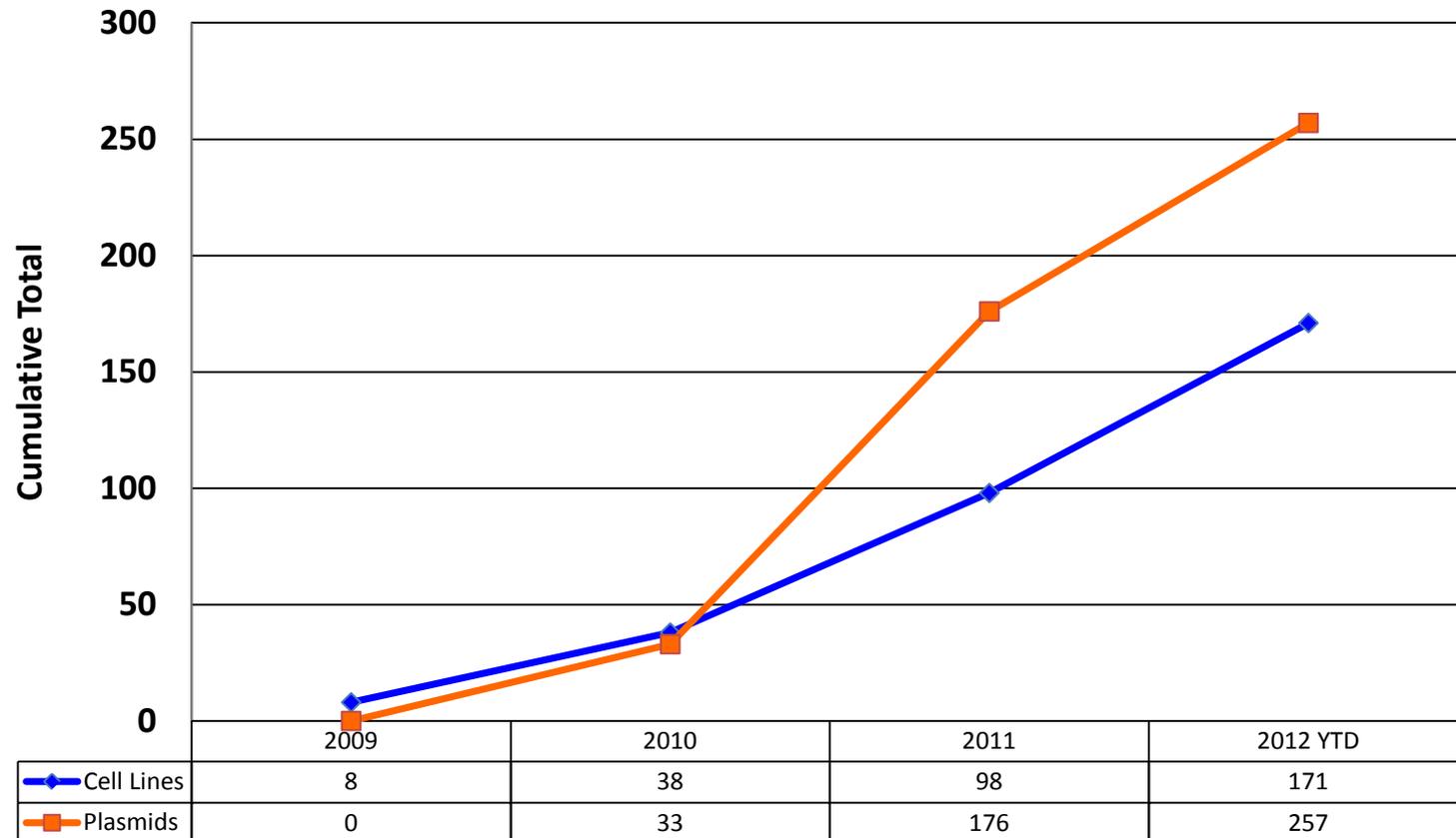
Add to Cart

Reagent Information	
Name	HEK293T
Reagent Type	Cell Line
Vector Classifications	Lentivirus Retrovirus
Keywords	
Cell Line Origin	Human Embryonic Kidney Cells
Short Description	Used for transient transfection of plasmids, including production of lentiviral and retroviral vectors.
Long Description	HEK293T cells are a human cell line that can be transfected with plasmid DNA at high efficiency. The cell line was derived from the HEK293 cell line by the addition of the SV40 large T antigen that has been shown to increase vector production of some viral vectors. It is commonly used for production of retroviral, lentiviral and other viral vectors using the transient transfection method.
Vector Map	
Sequence	
Grade	Suitable for use on Phase I Clinical Trials
Depositor	Cells were originally obtained from the ATCC
References	
Biosafety	This cell line has screened negative for viral and other contaminants. It is the policy of Indiana University to maintain cells of human origin under BL2 conditions but the level of containment required of any cell line or vector is at the discretion of the investigator's Institutional Biosafety Committee that may require a different level of containment.
Comments	As only a limited number of certified vials are available, the material will be distributed only to those investigators conducting federally funded development of clinical trial material. Grant number and specific aims should be provided to the Coordinating Center with cell line request. Individuals seeking this line for research purposes should obtain the cells through the ATCC (www.atcc.org).
Storage	Liquid Nitrogen
Availability	In Stock

Reagents dispensed over time

National Gene Vector Biorepository

Reagents Shipped To Date



Archiving Services

National Gene Vector Biorepository

- Over 13,000 samples from GLP Pharm/Tox studies
- Back-up of GMP grade vectors and cell lines
- Archive patient samples to comply with FDA requirements for post-trial monitoring

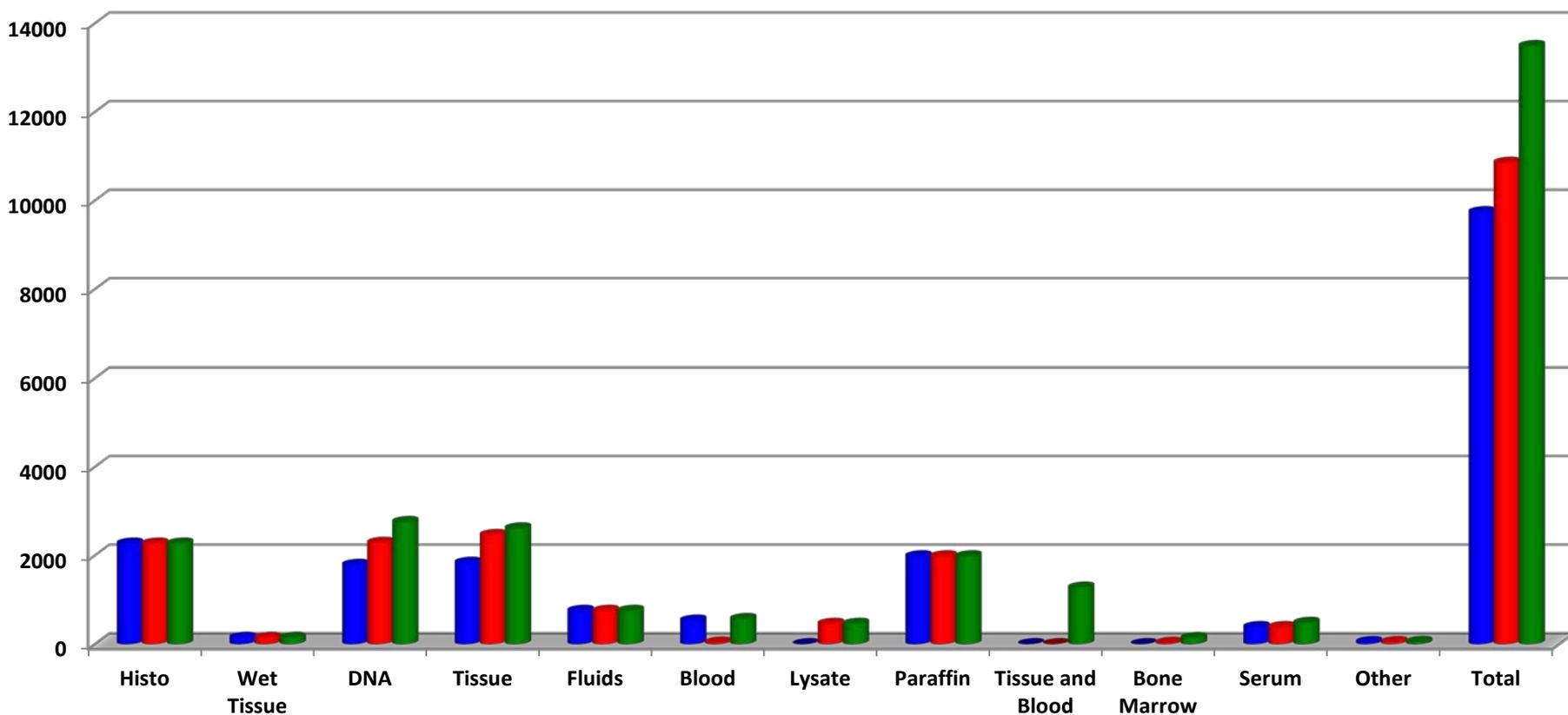


GLP Pharm/tox Archive

National Gene Vector Biorepository

GLP Pharm/Tox Archive

■ Total Jan 2009 ■ Total Jan 2010 ■ Current



Post-Trial Monitoring

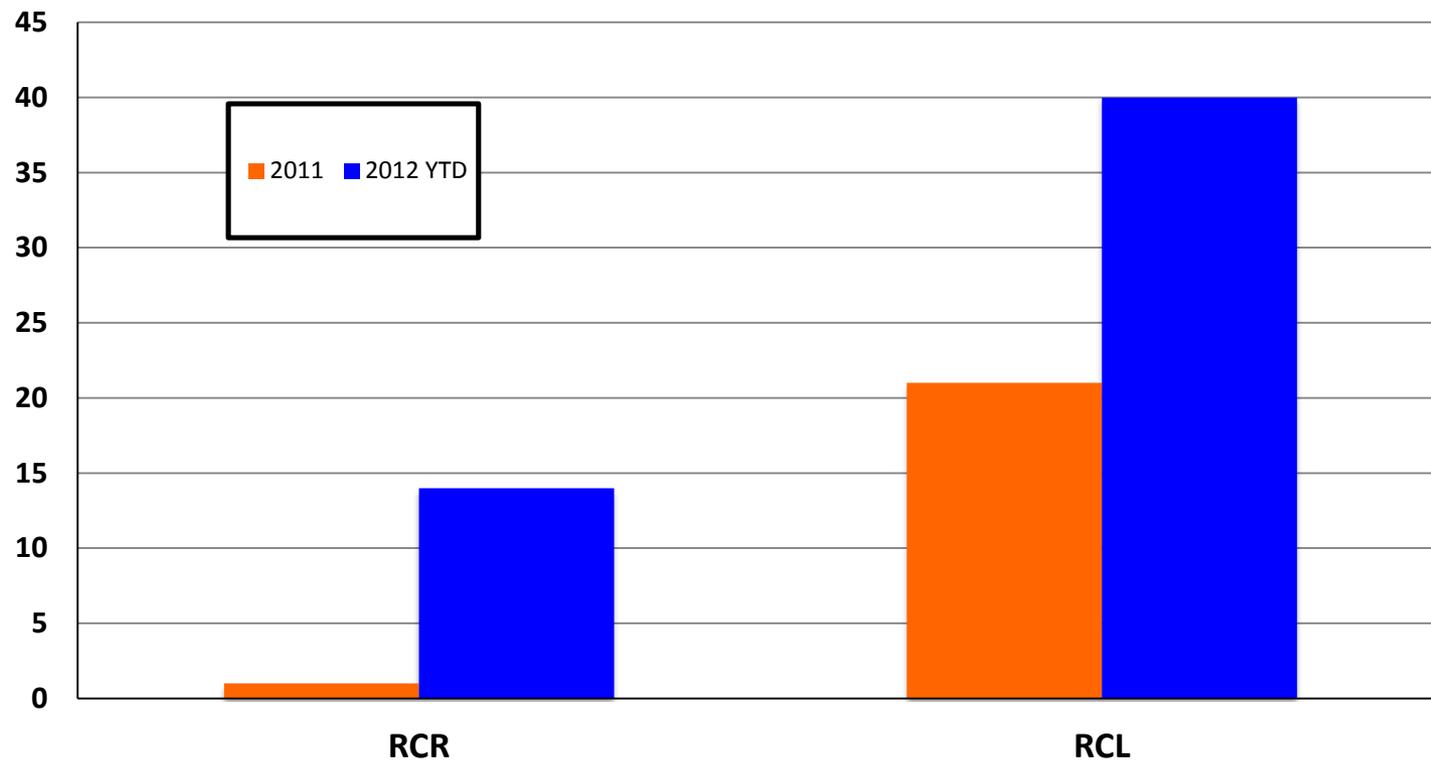
National Gene Vector Biorepository

- ▣ RCR and RCL testing on clinical cell products
- ▣ RCR and RCL testing of patient samples
- ▣ LAM-PCR or other insertion site analysis
- ▣ SeqMap 2.0 – a bioinformatics tool to help analyze LAM-PCR and other integration analysis.

RCR/RCL Biologic Assays

National Gene Vector Biorepository

Biologic Assays: Samples Tested



SeqMap

Submitted sequences are represented graphically

Chaos

Censor

BLAT

Each sequence is mapped to the genome, using the following three-step protocol:

DNA sequences are displayed, colored to match the graphical representation to aid in distinguishing its sections

original

vector

chr3

Proposed Insertion Site

TOPO_Start

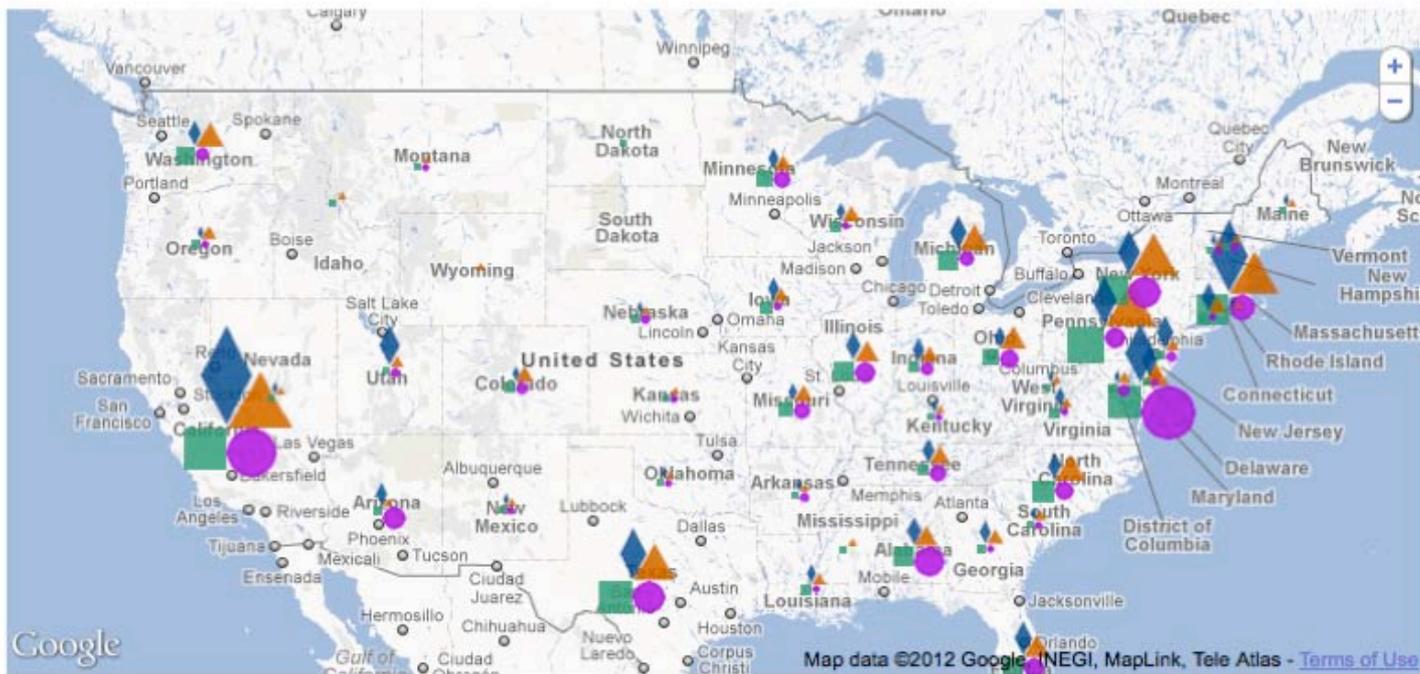
LTR_adaptorPrimer

LTR1

TOPO_End

Description (Edit Description)	Sequence
SEQ03-1.2	Original Sequence GCTTGGTCCGAGCTCGATCCACTAGTACGGCCCGCAGTGTGCTGGAATTCGCCCTCCATCCCTGCA
Sample (Edit Sample)	AAATGGCGTTACTGCAGCTAGCTTGCCAACTCAGGTGGGCTCTTCAGTAGTGTATCAACTGAGAT
SEQ03-1	AACCAGAAATCATTCTATATTTCTATATTTCCAGGGCAGGCCCCACAGTGAGGTGTTAGAACCTGGTCTCT
Comment (Edit Comment)	CCAGAGAATCACCACGGCTGCCNTAAGANGGGCAATTCTGCTGCAGATTTCNATCCACTGGGGGG
	GCNGCTCGAGCATGCATCTAAGGGGCCAANTTCGCCCTATAGTGAGTGGTATTACAATTCAGTGCNGT
	CNTTNTACACNNTCNTGACNGGAAAAAAA
BLAT Annotation (Full Summary)	
# Hits Found:	2
User Data	
Add User Data	
Calculation of Top UCSC Hit	Vector Removed Sequence
Gene Symbol:	Ev1
Gene Location:	upstream
Identity:	100.0
Strand:	-
Closest End (kbp):	109.4
Gene ID:	NM_007963
Human Ortholog:	EV11
Calculation of Top Ensembl Hit	
Gene Symbol:	Ev1
Gene Location:	intron 1
Identity:	100.0
Strand:	-
Closest End (kbp):	443.4
Gene ID:	NM_007963.1
Human Ortholog:	EV11
	Repeat Masked Sequence
	GCTTGGNN
	NN
	AACCAGAAATCATTCTATATTTCTATATTTCCAGGGCAGGCCCCACAGTGAGGTGTTAGAACCTGGTCTCT
	CCAGAGAATCACCACGGCTGCCNTAAGANGGGCAATTCTGCTGCAGATTTCNATCCACTGGGGGG
	GCNGCTCGAGCATGCATCTAAGGGGCCAANNN
	CNTTNTACACNNTCNTGACNGGAAAAAAA

Welcome to the Gene Therapy Data Map



- Funding** ⓘ
- Publications** ⓘ
- Patents** ⓘ
- NIH
- Medline
- USPTO
- NSF
- Clinical Trials** ⓘ

From year to year

Search by:

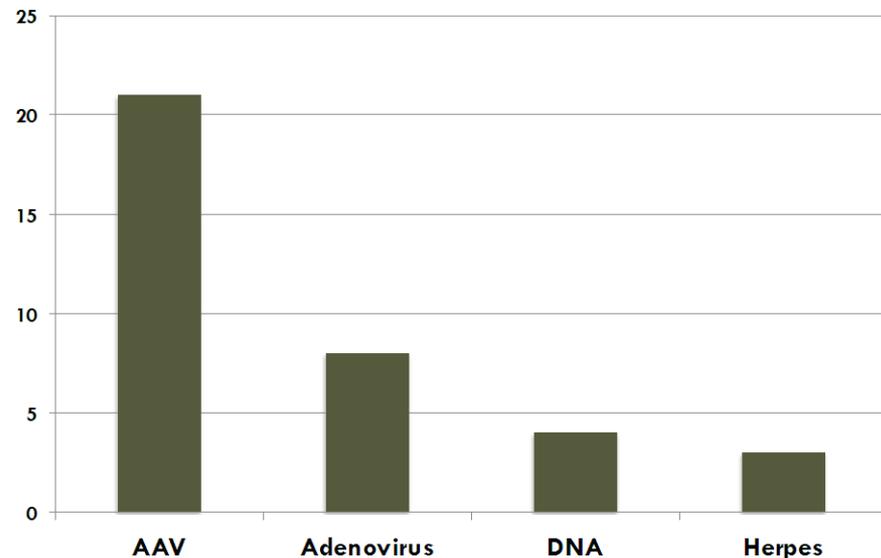


Maps	Detail	Data	About
<p>About</p> <p>Gene therapy seeks to offer new therapeutic options to patients suffering from a wide range of diseases. This work combines the insights into human illness illuminated by the human genome project with advances in gene transfer technology. The field is highly interactive involving basic scientists, engineers, bioethicists, and clinicians. As the methods of gene transfer are a new paradigm, the novel therapies are of significant interest to patients, regulators and the press.</p> <p>Please consult the National Gene Vector</p>			

Pharm/Tox Database

National Gene Vector Biorepository

- ▣ Detailed description of Pharm/Tox studies submitted to the FDA in support of gene therapy INDs
- ▣ Foster letters of cross-reference
- ▣ Educational tool



NGVB

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SeqMap
Database

rAAV Reference
Standard

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Toxicology Reports

Welcome to the Toxicology database. This area holds a summary of Toxicology studies that have been submitted to our database.

The information contained here is for public view. You may search for a study by selecting an option on the right. If you wish to see details of the data or share your own data then you would need to obtain a user account and log-in [click here](#). Once you have acquired your user name and password you can then enter your own study into the database by choosing "Submit a New Report, or you can view reports that you have already entered by choosing "View Your Reports". You can also view a detailed version of all the studies entered by selecting the search options.

Investigators wishing to obtain a letter of cross-reference from the Principal Investigator of a particular study may do so by emailing

Search for Reports by:

Vector System:

Select a vector system...

Animal Species:

Select a species...

Vector Grade:

Select a grade...

Clinical Indication:

Select an indication...

Route of Administration

Select a route...

Search Reports

[Clinical Trial Publications
Associated with Database
Toxicology Studies](#)

[Toxicology Database User
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Home > Toxicology Database > Toxicology Reports

Toxicology Reports

ository

Search parameters:

- **Animal Species:** Monkey

Query results:

1. Single Dose 6-Month Toxicity Study of Adeno-Associated Virus-Cystic Fibrosis (AAV-CFTR) Gene Vector in the Rhesus Monkey
2. Myocarditis Following Adeno-Associated Viral Gene Expression of Human Soluble TNF Receptor (TNFR1I.Fc) in Baboon Hearts.
3. Three Month Toxicity and Biodistribution Study of AAV2-SB-hRPE65 Following a Single Subretinal or Intravitreal Injection in Cynomolgus Monkeys.
4. One-week Toxicity and Biodistribution Study of AAV2-SB-hRPE65 Following a Single Subretinal Injection in Cynomolgus Monkeys.

Search for Reports by:

Vector System:

Select a vector system...

Animal Species:

Select a species...

Vector Grade:

Select a grade...

Clinical Indication:

Select an indication...

Route of Administration

Select a route...

Search Reports

Clinical Trial Publications
Associated with Database
Toxicology Studies

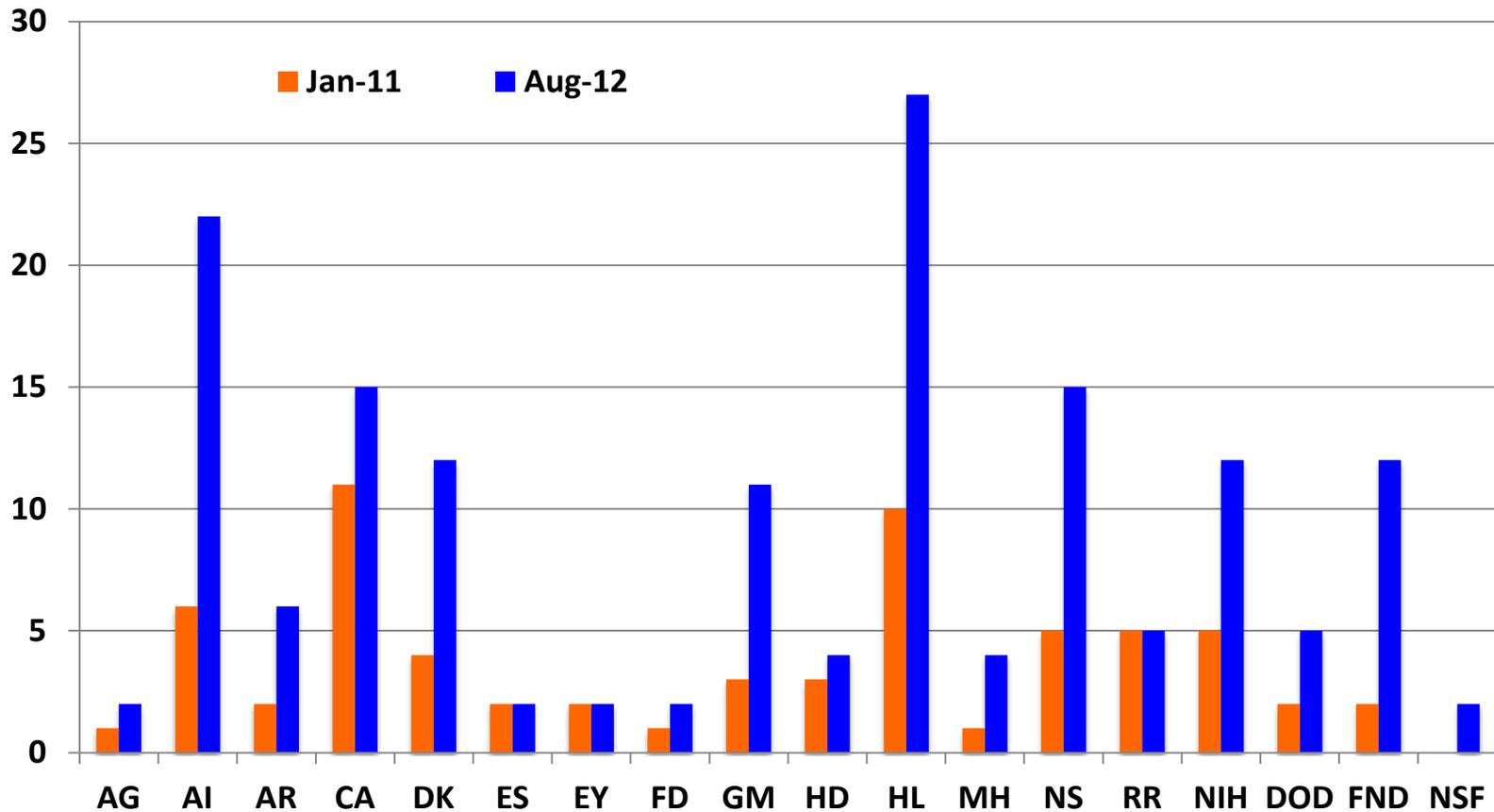
Toxicology Database User
Guide

Cost

Resource	Cost
Reagents	FREE to academic investigators
Pharm/Tox Database Info	FREE to all
Archiving Services	FREE to academic US investigators
SeqMap	FREE to all
Insertion Site (ex. LAM-PCR)	FREE to academic investigators
RCR/RCL Testing Post-Trial Monitoring	FREE to academic investigators

NGVB Supports 160 Extramural Grants

National Gene Vector Biorepository



DONATE REAGENTS !!!!!

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Not NGVB.org !!!!



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Summer League

The summer league has come upon us! After a short break, the new season comes with many new faces and new teams. Welcome to the new players!

Download the [2008 Summer League Schedule](#).



Congratulations to our
league champs!

A Pool: Team 3, Captain Mao Wang





Lorraine Matheson
Hongyu Gao, PhD
Aparna Jasti
Lisa Duffy
Lilith Reeves
Erol Cetinok
Daniela Bischof, PhD
Sue Koop
Jing Yao
Lina Segó
Alisha Auberry
Anna Leath
Aaron Shaw



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