Results from the ASHP Gene Therapy Survey

Susan Goodin, Pharm.D., FCCP, BCOP
Associate Director, Clinical Trials and Therapeutics
The Cancer Institute of New Jersey
Professor of Medicine
UMDNJ-Robert Wood Johnson Medical School

Survey Methods

- Administered via email invitation, linking participants to an online survey instrument
- Sent to 3,543 members recorded in by ASHP as Pharmacy Directors
- Launched November 19, 2008; closed December 5, 2008 (one reminder)
- 327 responses; 9% response rate
- Margin of error is ± 5% at the 95% confidence level

Respondent Characteristics

- Majority (73%) were Pharmacy Directors at institutions caring for both adult and pediatric patients (77%)
- Community Hospitals represented 58% of respondents; university hospitals represented 11%
- Size (# of beds) of the institution varied: 400 + (25%), 300-399 (10%), 200-299 (16%), 100-199 (16%), does not apply (10%)

Gene Therapy Trials are not infrequent and not increasing?

- True
- False

Participation in Gene Therapy Clinical Trials

Participated in the past	11%
Currently conducting trials	12%
Participation in the next 1 to 2 years	12%
None of the above	75%

n=327

Estimate of Gene Therapy Trials

Trials	Past 18 months	Next 1 to 2 years
None	42%	11%
1-3	36%	44%
4-6	7%	13%
7-10	3%	4%
11 or more	3%	7%
Not Sure	9%	21%
Responses	67	71

Types of Gene Therapy Trials

Cancer	66%
Blood cell disease	31%
Cardiovascular	31%
Genetic disease	15%
Neuro or movement condition	12%
Infection disease	10%
Respiratory disease	10%
Chronic pain	3%
Ocular disease	3%
Other	6%
Not sure	15%

n=?

The pharmacy department is responsible for storage of gene therapy products the majority of the time.

- True
- False

Where are gene therapy products stored?

- The most common storage is in the pharmacy (37%)
- Outside the pharmacy in a lab; with a study coordinator; and in the GTP facility were storage sites for 10%
- Both within and outside the pharmacy is storage site at some (16%) institutions
- Still planning storage (37%)

n=73

Available equipment for storage

Freezer – 80°C	83%
Freezer –20 °C	63%
Refrigerator (2 to 8°C)	61%
Liquid Nitrogen Tank	33%

n=46

Types of Vectors/Carriers

Viral	58%
Patients own cells	21%
Plasmids	19%
Bacteria	10%
Not sure	42%

n=67

Pharmacy Involvement

IRB process	75%
Product storage	64%
Product Preparation	65%
Product Disposal/Return	55%
Patient education	22%
Protocol development	30%
Other	13%
None	6%

Most pharmacy departments participating in gene therapy trials have dedicated biologic safety cabinets for preparation.

- True
- False

Use of Biological Safety Cabinet for Gene Product Preparation

- Multipurpose 48%
- Dedicated 22%
- Not sure 30%

n=63

Pharmacy Department

	Yes	No
Provides staff training on gene therapy (n=62)	40%	60%
Has gene therapy policies and procedures (n=63)	33%	67%
Is planning to develop policies and procedures within the next 12-24 months (n=41)	63%	37%

Who prepares gene therapy?

- Staff with specific training 48%
- Any staff with hazardous training 25%
- IDS pharmacist 5%
- Not sure 22%

n=60

Challenges preventing participation in gene therapy trials

- Lack of training and staff education
- Lack of personnel
- Lack of capital equipment
- Lack of policies and procedures
- Staff fear of handling gene therapy

n=290

ASHP Gene Therapy Survey Conclusions

- Participation in gene therapy trials is increasing
- Cancer, blood diseases, and cardiovascular disease are the most common areas of gene therapy research reported at responding institutions
- Storage of gene therapy products frequently occurs in the pharmacy and most have appropriate equipment
- Staff training and policies are lacking related to gene therapy
- Patient education is not a frequent role for the pharmacy department in gene therapy clinical trials