Results from the ASHP Gene Therapy Survey

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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%

Estimate of Gene Therapy Trials

<table>
<thead>
<tr>
<th>Trials</th>
<th>Past 18 months</th>
<th>Next 1 to 2 years</th>
</tr>
</thead>
<tbody>
<tr>
<td>None</td>
<td>42%</td>
<td>11%</td>
</tr>
<tr>
<td>1-3</td>
<td>36%</td>
<td>44%</td>
</tr>
<tr>
<td>4-6</td>
<td>7%</td>
<td>13%</td>
</tr>
<tr>
<td>7-10</td>
<td>3%</td>
<td>4%</td>
</tr>
<tr>
<td>11 or more</td>
<td>3%</td>
<td>7%</td>
</tr>
<tr>
<td>Not Sure</td>
<td>9%</td>
<td>21%</td>
</tr>
<tr>
<td>Responses</td>
<td>67</td>
<td>71</td>
</tr>
</tbody>
</table>

n=327
Types of Gene Therapy Trials

<table>
<thead>
<tr>
<th>Condition</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cancer</td>
<td>66%</td>
</tr>
<tr>
<td>Blood cell disease</td>
<td>31%</td>
</tr>
<tr>
<td>Cardiovascular</td>
<td>31%</td>
</tr>
<tr>
<td>Genetic disease</td>
<td>15%</td>
</tr>
<tr>
<td>Neuro or movement condition</td>
<td>12%</td>
</tr>
<tr>
<td>Infection disease</td>
<td>10%</td>
</tr>
<tr>
<td>Respiratory disease</td>
<td>10%</td>
</tr>
<tr>
<td>Chronic pain</td>
<td>3%</td>
</tr>
<tr>
<td>Ocular disease</td>
<td>3%</td>
</tr>
<tr>
<td>Other</td>
<td>6%</td>
</tr>
<tr>
<td>Not sure</td>
<td>15%</td>
</tr>
</tbody>
</table>

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%)

Available equipment for storage

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

Types of Vectors/Carriers

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

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%

Pharmacy Department

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

Who prepares gene therapy?

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

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