

# The National Hemophilia Foundation

American Society of Gene Therapy  
Stakeholders Meeting



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Chief Executive Officer

# The National Hemophilia Foundation

Q. Why is there a dearth of funding for gene therapy?

A. i. Relatively few successes.

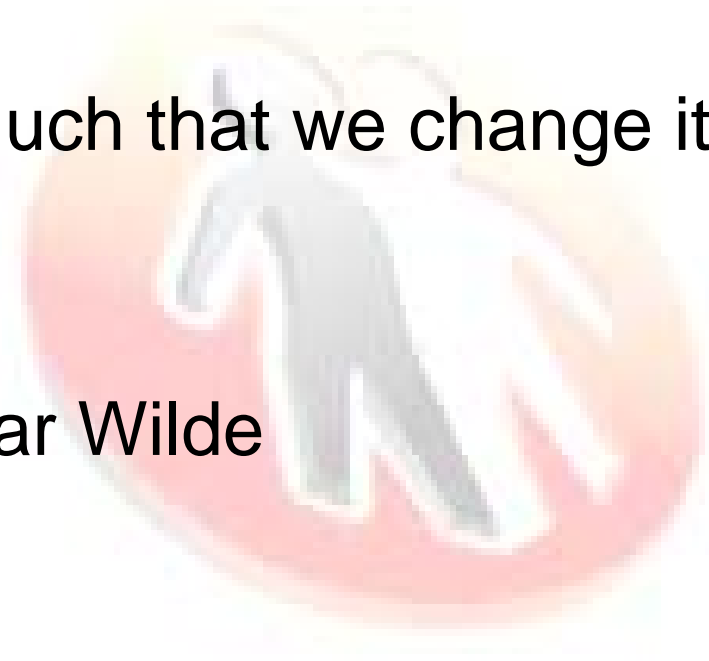
ii. Certain highly publicized cases of morbidity and mortality: T-cell leukemia, liver toxicity which has led, in certain cases, to death.

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iii. Gene therapy is not “popular” anymore, it’s not sexy.

“We hate fashion so much that we change it every six months”

- Oscar Wilde



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How can Voluntary Health Agencies help to accelerate gene therapy?

1. VHAs can directly fund more gene therapy research.
2. VHAs can advocate for more gene therapy research funding by other organizations, e.g., NIH, DoD, big pharma, biotech.

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Can VHAs pool their resources to fund more adequately gene therapy research?

Can they create a funding consortium to support gene therapy research and clinical trials?

Why not??

