Clinical Care Strategies for GENE THERAPY

RAPID RECAP

LEARNING OBJECTIVES

- Outline key components of referring, preparing, and administering therapies for eligible patients
- Implement a personalized approach to hemophilia management based on outcomes of gene therapy (e.g., resulting factor level and change over time)
- Follow short- and long-term monitoring requirements
- Implement modifications to the multidisciplinary care approach necessary for gene therapy

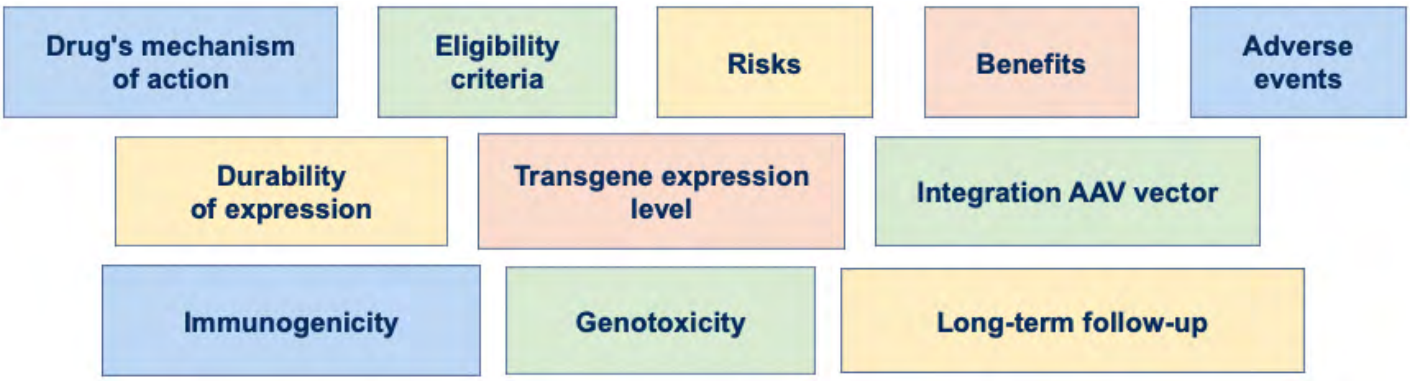
KEY COMPONENTS OF ADMINISTERING GENE THERAPY

Healthcare specialist/professionals[1,2]:

- Provide **appropriate, personalized guidance and support** to ensure that patients fully understand the options available, **benefits, risks, adverse events, impact on quality of life** and **eligibility**

Patient education[3-6]:

- Patients require comprehensive information on the following topics prior to receiving gene therapy:



- Supporting patients' **decision-making autonomy** is **extremely important**[4-6]

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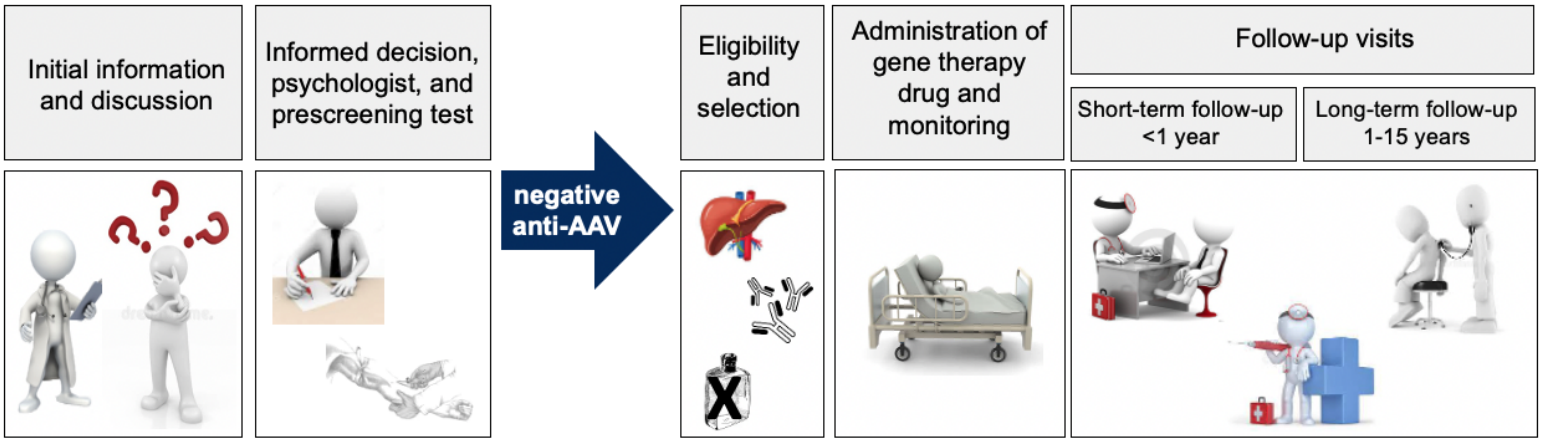
CLINICAL CARE PATHWAY[1-3]



ROLE OF THE HEPATOLOGIST

- Selection of candidates: hepatological evaluation[7-9]
  - Liver enzyme tests
  - Liver ultrasound and FibroScan® to check for signs of damage or scarring
- Management of the risk of short-term hepatotoxicity (< 3-6 months)*
- Management of the risk of long-term hepatotoxicity (≥ 6 months)*

*these time cut-offs are based on Dr. La Mura's clinical experience

ROLE OF THE NURSE[10,11]

- Organize the setting
- Manage patient expectations
- Support decision-making
- Help coordinate the gene therapy journey
- Manage the operations
- Enter items into electronic medical records
- Facilitate infusion and administration of gene therapy
- Recognize and mediate TEAEs

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MONITORING POST-INFUSION WITH GENE THERAPY[7-9,11]

DAYS TO WEEKS

- Identify and mitigate TEAEs
- Coordinate monitoring with follow-up center
- If indicated, start patient on immunosuppressive treatment
- Confirm patient understands importance of follow-up
- Encourage participation in a registry
- Assess factor levels and bleeding events

WEEKS TO MONTHS

- If indicated, continue immunosuppressive treatment
- Assess factor levels and bleeding events

MONTHS TO YEARS

- Serve as a source for any patient questions
- Conduct annual lab and safety assessment
- Assess factor levels and bleeding events

ASSAYS USED TO MEASURE EFFICACY[12-14]

Measures expression of the factor transgene

- Vector genome copy number

Measures factor activity:

- One-stage clotting assay
- Chromogenic assay

Measures quantity of factor:

- Antigen assay

Measures factor inhibitors:

- Bethesda assay

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KEY TAKEAWAYS

- Multidisciplinary care model is key to managing patients with gene therapy
- Patients with hemophilia need to be fully informed regarding the potential benefits and risks of gene therapy
- Information, education, and discussion about gene therapy should be provided by physicians, nurses, and patient associations
- Increase in liver enzymes needs further investigation and knowledge of management
- Data collection post-infusion is mandatory
- Safety must remain the primary objective

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