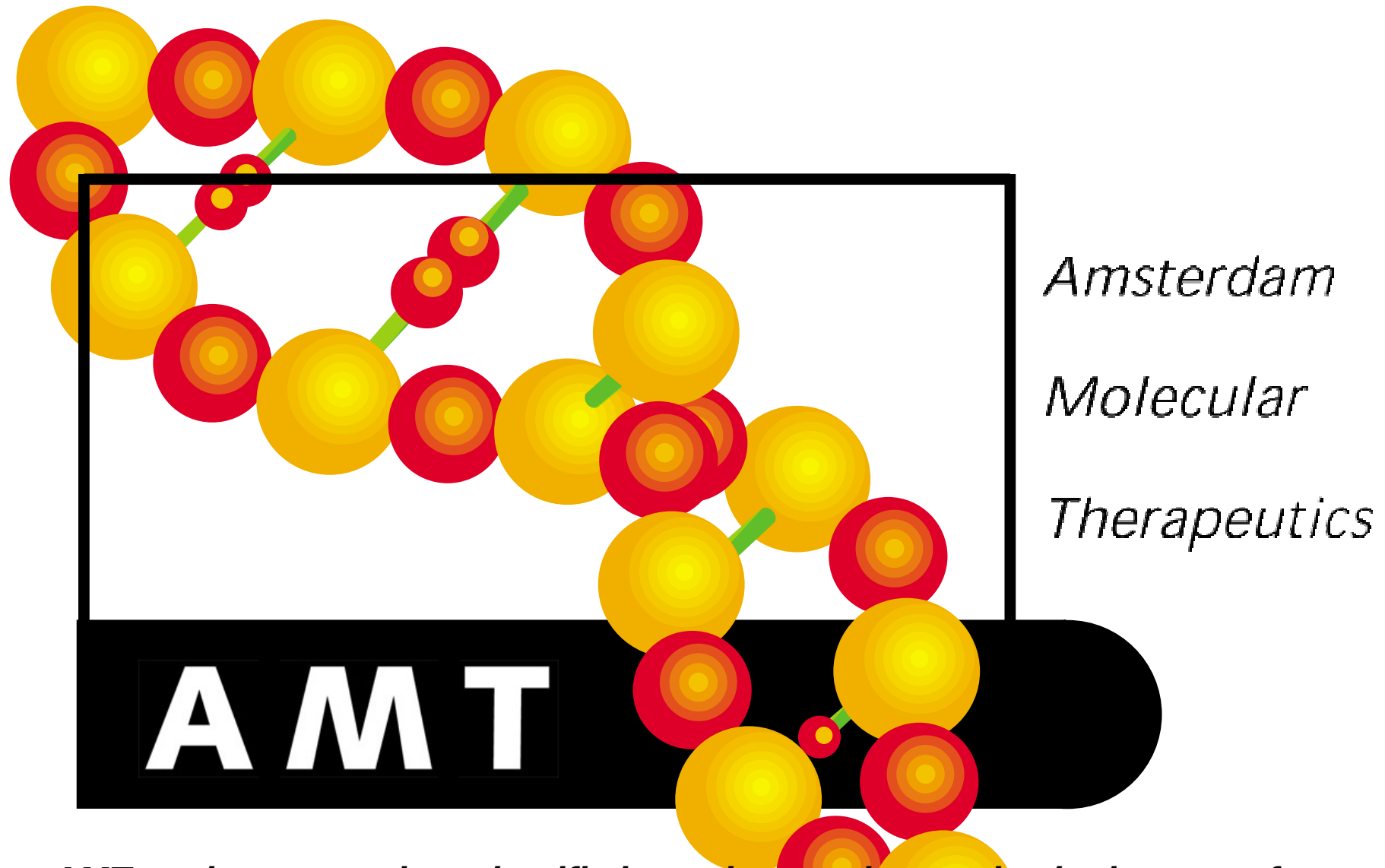


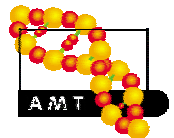


AMT aspires to use its scientific know-how and expertise in the area of gene therapy in order to successfully develop innovative treatments for patients with serious inherited, metabolic, cardio-vascular or central nervous disorders.

Forward looking statements

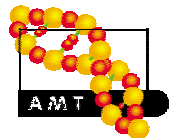
Certain statements in this presentation are “forward-looking statements” including those that refer to management's plans and expectations for future operations, prospects and financial condition. Words such as “strategy,” “expects,” “plans,” “anticipates,” “believes,” “will,” “continues,” “estimates,” “intends,” “projects,” “goals,” “targets” and other words of similar meaning are intended to identify such forward-looking statements. Such statements are based on the current expectations of the management of Amsterdam Molecular Therapeutics only. Undue reliance should not be placed on these statements because, by their nature, they are subject to known and unknown risks and can be affected by factors that are beyond the control of AMT. Actual results could differ materially from current expectations due to a number of factors and uncertainties affecting AMT's business, including, but not limited to, the timely commencement and success of AMT's clinical trials and research endeavors, delays in receiving U.S. Food and Drug Administration or other regulatory approvals (i.e. EMEA, Health Canada), market acceptance of AMT's products, effectiveness of AMT's marketing and sales efforts, development of competing therapies and/or technologies, the terms of any future strategic alliances, the need for additional capital, the inability to obtain, or meet, conditions imposed for required governmental and regulatory approvals and consents.

AMT expressly disclaims any intent or obligation to update these forward-looking statements except as required by law. For a more detailed description of the risk factors and uncertainties affecting AMT, refer to the prospectus of AMT's initial public offering on June 20, 2007, and AMT's reports filed from time to time with Euronext Amsterdam N.V.

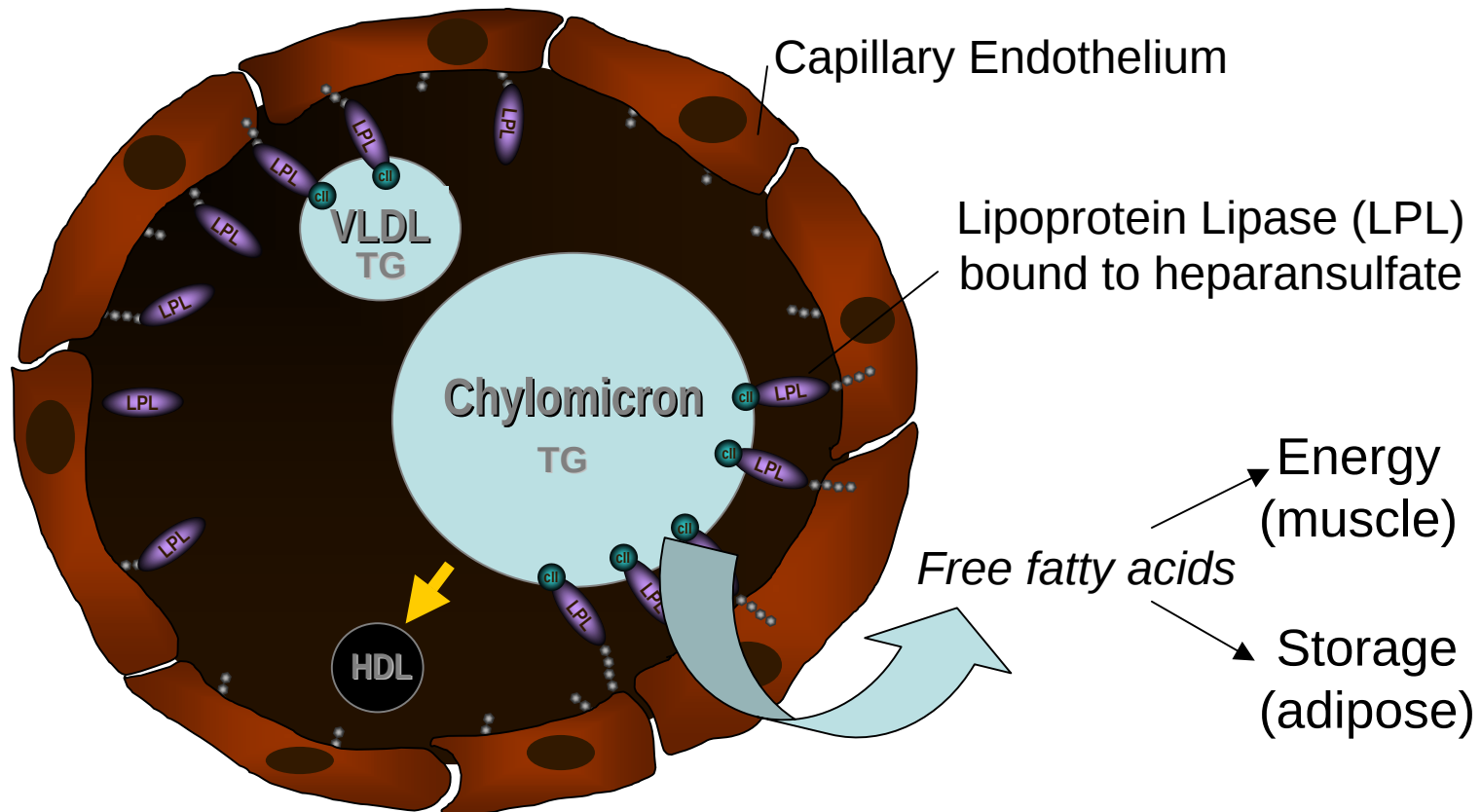


Biodistribution/shedding of AAV1-LPL^{S447X} Vector Sequences after Intramuscular Administration to Lipoprotein Lipase Deficient Patients

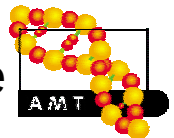
Janneke Meulenberg



Lipoprotein Lipase (LPL)



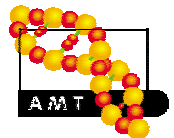
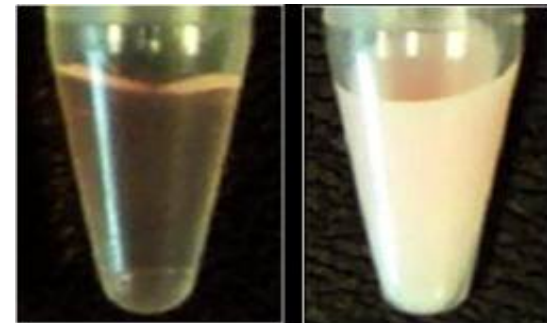
- Synthesized in muscle and adipose tissue
- Transported and bound to the capillary endothelium
- Breaks down triglycerides (TG) in Chylomicrons and VLDL
- Free fatty acids absorbed by underlying tissues for energy & storage



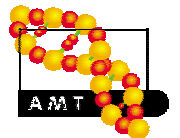
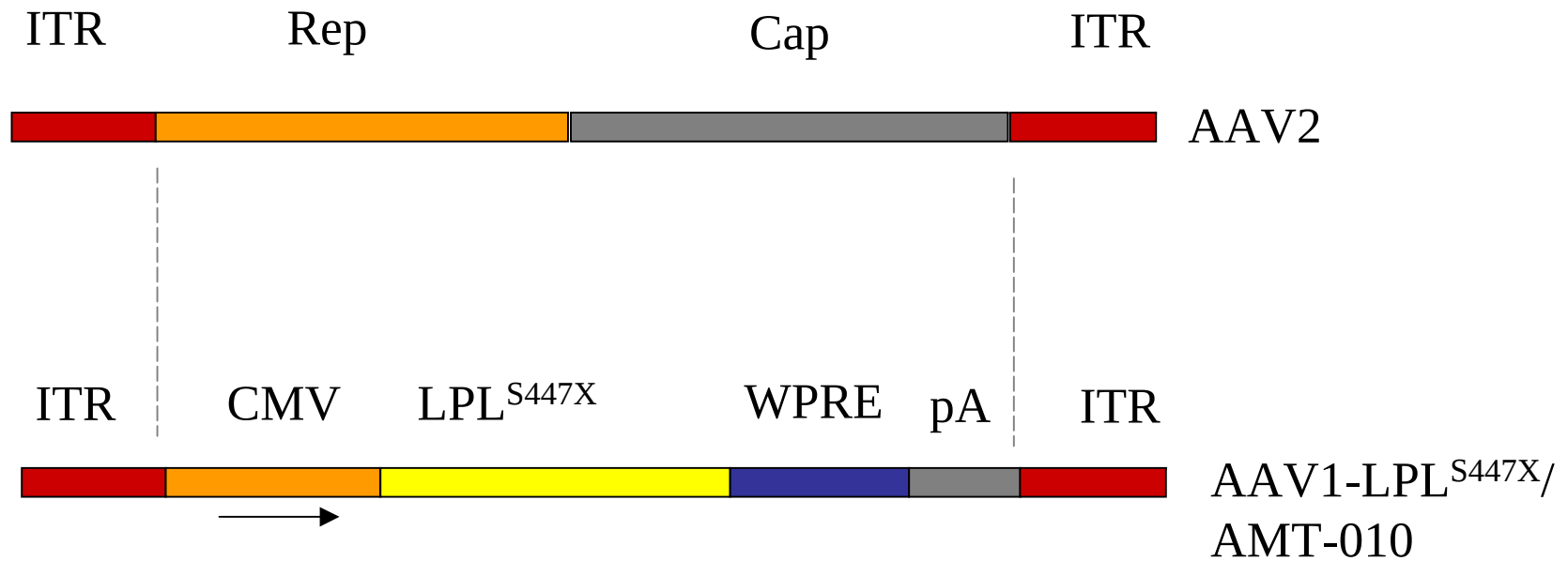
LPL deficiency

- Type I hypertriglyceridemia: rare autosomal recessive disorder caused by mutations in the LPL gene
- Clinical phenotype:
 - Hypertriglyceridemia
 - Abdominal pain
 - Acute pancreatitis (potentially lethal)
- Current treatment for LPL deficiency is ineffective

Normal plasma LPL deficient plasma

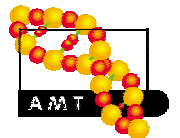


AAV1-LPL^{S447X} vector



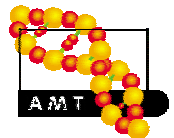
AMT-010 Gene Therapy Study

- Open label, dose escalation study
- Intramuscular administration of single dose of AMT-010 in multiple sites
- 12 weeks evaluation, 5 year long-term follow-up
- 4 patients injected with 1×10^{11} gc/kg,
- 4 patients injected with 3×10^{11} gc/kg



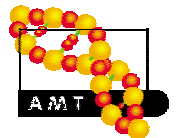
Biodistribution data generated in mice and cats

- Design of studies discussed with US and Dutch regulatory authorities
- Mice:
 - Dose 1×10^{11} and 1×10^{13} gc/kg = 100 fold higher than starting dose in clinical study
 - Time points: day 8, 29, 91
 - N=5/group
- Cats (N=5): Semen, liver and muscle tested from animals used in efficacy studies (between 1×10^{11} and 2×10^{12} gc/kg)



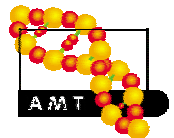
Results from biodistribution studies in mice and cats

- Dose-dependent leakage of vector into the circulation
- Rapid clearance from blood
- Persistence (up to 90 days) of vector in muscle and lymph nodes
- Very little spread to gonads



Regulatory approval in The Netherlands

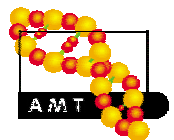
- Competent authority (patient safety): “CCMO”
- Approval for deliberate release into the environment: “Bureau GGO/VRM”
- Bureau GGO/VRM advised by a special recombinant advisory committee (COGEM)



Important considerations for environmental risk assessment of AMT-010 gene therapy

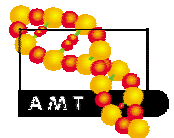
- AAV is a non-replicating virus and needs adenovirus for replication
- AMT-010 vector is devoid of AAV genes
- AMT-010 vector production process is designed as such that replication competent AAV is not produced (proven by experimental data !)
- Rep gene + Cap gene + LPL^{S447X} transgene can not be packaged in one single particle

Conclusion: chances for complementation or recombination are extremely low



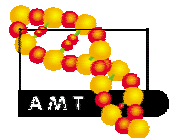
Preventive measures proposed in the clinical trial application

- Negative pressure in the hospital room during injection
- Hospital staff obliged to wear glasses, masks, and gloves to prevent exposure to AMT-010
- Disposables incinerated and equipment decontaminated
- Females should use barrier contraception until 12 weeks after administration
- Males should use barrier contraception until 3 consecutive samples taken at least 75 days after injection are negative.
- Monitoring of serum, saliva, urine and semen for AMT-010 vector sequence by Quantitative PCR



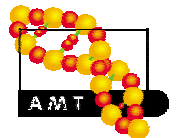
Preventive measures requested by the Dutch regulatory authorities

- Patients with active Helper virus infections (i.e. Adeno or Herpes) should be excluded from the study
- Females should use barrier contraception until 12 weeks after administration
- Males should use barrier contraception until 3 consecutive samples taken at least 75 days after injection are negative.
- Injected patients should not donate tissue for transplantation purposes



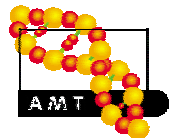
Clinical trial application in Canada

- Patient safety and environmental risk assessment reviewed by one and the same authority: Health Canada
- Requested to add safety measures as appendix to the protocol and add instructions for patients in the informed consent
- Safety measures incorporated in the clinical trial application were the same as for Dutch study. The requirement for a negative pressure during the injection was deleted.



Preventive measures requested by Health Canada

- Instructions incorporated in the informed consent:
 - Avoid close contact such as kissing
 - Practice effective barrier methods of contraception until three consecutive semen samples, taken at least 75 days after administration, are negative for AMT-011 vector DNA
 - Avoid coming in contact with anyone who has an acute adenovirus or herpes virus infection
 - Keep your tooth brush and eating utensils separate
 - Wash your hands frequently, especially after using the washroom, blowing your nose or coughing
 - If surfaces such as the toilet seat come in direct contact with stool or urine, wipe clean with chlorine bleach



Concluding remarks

- Environmental risk of AMT-010 gene therapy and AAV-based gene therapies in general is very low
- Requirements for preclinical and clinical shedding data and risk management procedures should be balanced against this low risk
- Requirements should be defined on a case by case basis
- Platform studies should be accepted for environmental risk assessment of gene therapies

