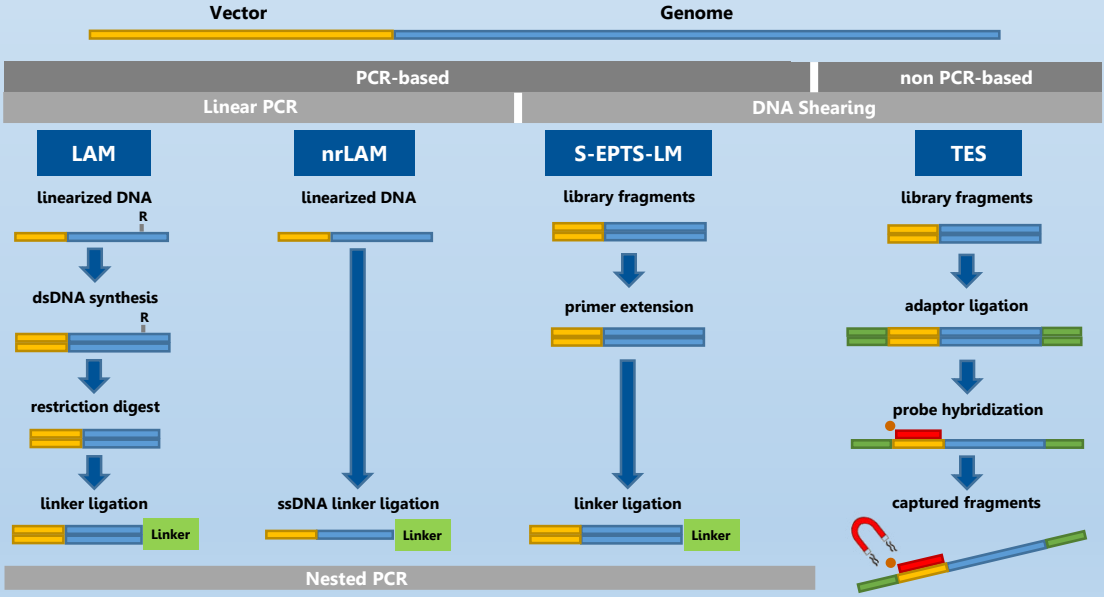
Towards Safer Therapies

Development of Innovative Research Concepts and Platforms

Our Integration Site Analysis Tool Suite



Sequencing and in-house Bioinformatic Analysis

Schematic outline of our integration site (IS) analysis next generation sequencing (NGS) tool suite:

- **Linear Amplification-Mediated (LAM) PCR** managed the comprehensive and quantitative IS analyses in a lot of worldwide gene therapy trials. The initial linear PCR step combined with optimized further reaction steps guarantees the successful IS retrieval in minimal tissue samples.
- **Non-Restrictive (nr) LAM PCR** variant avoids the use of restriction enzymes and thus guarantees the detection of emerging and dominant clones present in the analyzed samples.
- **Shearing Extension Primer Tag Selection Ligation-Mediated PCR (S-EPTS/LM-PCR)** matches up 'old (LM) and new (NGS)' to make use of sheared fragment lengths for clonal quantification. The implementation of additional unique molecular identifiers (UMI; validation is ongoing) avoids distorted clone measurements due to high depth NGS saturation.
- **Target Enrichment Sequencing (TES)** goes beyond quantitative IS analyses and measures vector genome stability and vector copy number (VCN) in the analyzed DNA samples.

References:
Schmidt M et al. Nature Methods 4.12 (2007): 1051-1057.
Gabriel R et al. Nature Medicine 15.12 (2009): 1431-1436.
Paruzynski A et al. Nature Protocols 5.8 (2010): 1379-1395.
Schmidt M et al. Human Gene Therapy 12.7 (2001): 743-749.

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