

Programs in R+D



| Program | Payload | Delivery | Indication | Gene | Discovery | Pre-Clinical | Phase 1 | Phase 2/3 |
|------------|---------|----------|---------------|-------|-----------|--------------|---------|-----------|
| CNS | | | | | | | | |
| SHP-101 | tRNA | AAV | Rett Syndrome | MECP2 | | | | |

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| Multi-Organ | | | | | | | | |
| SHP-111 | tRNA | AAV | Cystic Fibrosis | CFTR | | | | |

| Program | Payload | Delivery | Indication | Gene | Discovery | Pre-Clinical | Phase 1 | Phase 2/3 |
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|------------|------|-----|-------------|-------|--|--|--|--|
| CNS | | | | | | | | |
| SHP-201 | ADAR | AAV | Parkinson's | SNCA | | | | |
| SHP-202 | ADAR | AAV | Parkinson's | LRRK2 | | | | |
| SHP-231 | ADAR | AAV | Alzheimer's | APP | | | | |
| SHP-232 | ADAR | AAV | Alzheimer's | Tau | | | | |

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|------------|------|-----|--------------|--------|--|--|--|--|
| PNS | | | | | | | | |
| SHP-241 | tRNA | AAV | Chronic Pain | Nav1.7 | | | | |
| SHP-251 | ADAR | AAV | CMT1A | PMP22 | | | | |

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|--------------|------|-----|------|----------|--|--|--|--|
| Liver | | | | | | | | |
| SHP-221 | ADAR | RNA | AATD | SERPINA1 | | | | |

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|------------|------|-----|-------------------|-------|--|--|--|--|
| Eye | | | | | | | | |
| SHP-261 | ADAR | RNA | Stargardt Disease | ABCA4 | | | | |

| Program | Campaign | Route of Administration | Payload | Tissue Tropism | Capsid Discovery | Pre-Clinical | Phase 1 | Phase 2/3 |
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| SHP-301 | AAV5-id | Systemic | mRNA - RNAswitch | CNS | | | | |
| SHP-302 | AAV5-id | Systemic | mRNA - RNAswitch | Liver | | | | |
| SHP-303 | AAV5-id | Systemic | mRNA - RNAswitch | Muscle | | | | |
| SHP-304 | AAV5-id | Intravitreal | mRNA - RNAswitch | Retina | | | | |
| SHP-311 | AAV9-id | Systemic | mRNA - RNAswitch | CNS / Multiple | | | | |
| SHP-312 | AAV9-id | Intrathecal | mRNA - RNAswitch | CNS | | | | |