

# Case Study



## Regulatory Success for a US-Based Gene Therapy Company in the EU

### Challenge

A small, US-based specialty pharmaceutical company was focused on developing Advanced Therapy Medicinal Products (ATMPs) for rare genetic diseases. Their product portfolio included gene therapy solutions for inherited neurological and metabolic syndrome disorders. The company faced several challenges in navigating the complex regulatory landscape of the European Union (EU) due to its lack of an EU-based legal entity. The specific challenges included:

#### Regulatory Complexity:

- Managing the regulatory requirements for ATMPs in the EU, including classification, designation, and interactions with EU regulatory agencies.

#### Submission Documentation:

- Developing comprehensive and compliant submission documentation required for regulatory approval.

#### Strategy Development:

- Creating and implementing an effective regulatory strategy for multiple products without direct representation in the EU.

### Solution

To address these challenges, the company engaged tranScrip to act as their EU Regulatory Affairs (RA) Department. tranScrip provided the following solutions:

#### Regulatory Leadership:

- Assumed responsibility for all EU regulatory activities on behalf of the client, effectively functioning as the company's EU RA Department.

#### Documentation and Submission:

- Led the preparation and development of all required submission documentation, working closely with the client to ensure accuracy and compliance.

#### Agency Interaction:

- Managed all interactions with EU regulatory agencies, ensuring clear and effective communication and guidance throughout the regulatory process.

#### Regulatory Strategy:

- Developed and executed a comprehensive regulatory strategy for the client's products, addressing classification, designation, and other regulatory requirements.

#### SME Status:

- Assisted the company in obtaining EMA Small and Medium-sized Enterprise (SME) status, which provided access to various fee incentives and regulatory support mechanisms.



## Outcome

tranScrip's engagement led to significant achievements for the client:

### EMA SME Status:

- The company was successfully appended to the tranScrip EMA SME status, enabling access to fee incentives and regulatory benefits.

### ATMP Classifications:

- Achieved ATMP classifications for four of the company's products, validating their regulatory path for gene therapy in the EU.

### Orphan Medicinal Product Designation:

- Secured EU Orphan Medicinal Product Designation for two products, recognising their potential for treating rare diseases and granting additional regulatory benefits.

### Scientific Advice Meetings:

- Successfully completed Scientific Advice meetings with key EU member states:
  1. Finland for one product.
  2. Germany for two products, facilitating valuable feedback and guidance on product development and regulatory strategy.

## Conclusion

By leveraging tranScrip's expertise and comprehensive approach to EU regulatory affairs, the US-based company effectively overcame the challenges of entering the EU market.

The achievements of ATMP classifications, Orphan Medicinal Product Designations, and successful Scientific Advice meetings not only advanced their product development but also positioned the company for future success in the European market.

The successful attainment of EMA SME status further underscored the effectiveness of tranScrip's strategy, providing the client with a solid foundation for ongoing regulatory compliance and market access in the EU.

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