



EMA roundtable

15 years supporting SMEs

Orchard's experience

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Orchard Therapeutics Medium Size SME

Ex-vivo gene therapy
228 employees (Nov. 2020)

| Disorders | Diseases | Preclin | Clinical | MAA | Market | ATMP | ODD | PRIME |
|---------------------------------------|-----------|---------|----------|-----|--------|------|-----|-------|
| Neurometabolic / Neurodegenerative | MLD | | | X | | X | X | |
| | MPS-I | | X | | | X | X | X |
| | MPS-III A | | X | | | X | X | |
| | MPS-III B | X | | | | X | | |
| | FTD | X | | | | X | | |
| Immunological | ADA-SCID | | | | X | X | X | |
| | WAS | | X | | | X | X | |
| | X-CGD | | X | | | X | X | |
| | CROHN'S | X | | | | X | | |
| Blood | TDT | | X | | | X | X | X |

Orchard Therapeutics

Experience with SME support from EMA

| | Development | Registration | Post-MA lifecycle |
|--|--|---|----------------------------------|
| SME office support | <p>[2018] Proposed to organize a business pipeline meeting</p> <ul style="list-style-type: none"> ➤ Allowed Orchard to have an early dialogue with the Agency on our platform approach ➤ Allowed discussing scientific and regulatory issues seen across programs (CMC, NC and Clinical) | | |
| | <p>[2018; 2019] Facilitated joint meetings with the PRIME Coordinator to discuss Orchard’s proposal to request PRIME designation for development programmes.</p> <ul style="list-style-type: none"> ➤ Constructive meetings which helped with resource planning ➤ Also useful to inform any future requests for PRIME eligibility | | |
| | <p>[2020] Facilitated a request for Parallel Scientific Advice (PSA) for one clinical development program</p> <ul style="list-style-type: none"> ➤ Office acted as a link between Orchard and lead PSA coordinator at EMA. ➤ Contact with the PSA coordinator has proven invaluable. | | |
| | <p>[2020] Provided advice regarding the rules to allow a further renewal of the SME qualification in 2021 (so-called ‘two year’ rule).</p> | | |
| Translations assistance | | Product information for all EU languages | |
| Fees reduction (Orphan / PRIME) | Scientific advice (100%) | Inspection (100%) MAA fee (100%) | Variations & PV (40%) |