Inspection report on compliance with HTA licensing standards
Inspection date: **6 June 2024**

Vertex Pharmaceuticals Limited
HTA licensing number 12374

Licensed under the Human Tissue Act 2004

Licensed activities

Area	Storage of relevant material which has come from a human body for use for a scheduled purpose	Removal from the body of a deceased person (otherwise than in the course of an anatomical examination or post-mortem examination) of relevant material of which the body consists or which it contains, for use for a scheduled purpose other than transplantation
Vertex Pharmaceuticals Limited	Licensed	Not licensed

Summary of inspection findings

The HTA found the Designated Individual (DI) and the Licence Holder (LH) to be suitable in accordance with the requirements of the legislation.

Although Vertex Pharmaceuticals Limited ('the establishment') was found to have met the majority of the HTA's standards, three minor shortfalls were identified – these were against Governance and quality system (GQ) standards, relating to procedures, staff training and risk assessments.

The HTA has assessed the establishment as suitable to be licensed for the activities specified, subject to corrective and preventative actions being implemented to meet the shortfalls identified during the inspection.

Compliance with HTA standards

Minor shortfalls

Standard	Inspection findings	Level of shortfall
GQ1 All aspects of the establishments work are governed by documented policies and procedures as part of the overall governance process		
a) Ratified, documented and up-to-date policies and procedures are in place, covering all licensable activities.	Overarching documents provide staff with guidance on the management of human tissue, including receipt, storage, transport and disposal. However these did not provide sufficient detail for research groups to follow in a step-wise fashion. Furthermore, the research groups did not have local procedures in place, covering receipt and storage of tissue.	**Minor**

GQ3 Staff are appropriately qualified and trained in techniques relevant to their work and are continuously updating their skills		
b) There are documented induction training programmes for new staff.	There was no documented induction training for security staff who have responsibility for attending to critical storage alarms and notifying staff working under the licence.	**Minor**

GQ6 Risk assessments of the establishment's practices and processes are completed regularly, recorded and monitored		
a) There are documented risk assessments for all practices and processes requiring compliance with the HT Act and the HTA's Codes of Practice.	Risk assessments do not adequately address how risks to human tissue have been mitigated and the risk assessment to support the import of relevant material was incomplete.	**Minor**

Advice

The HTA advises the DI to consider the following to further improve practices:

Number	Standard	Advice
1.	C1(a)	The establishment's Consent procedure states that it is the establishment's responsibility to agree the process for withdrawal of consent with the supplier of tissue samples, prior to receiving samples. As this is not current practice, the DI is advised to review this paragraph and revise it accordingly.

2.	GQ2(a)	To gain wider assurances, the DI is advised to consider extending the range of audits to include coverage of processes and procedures; for example, staff undertaking a specific task.
3.	GQ5(a)	The DI should consider adding incident types to the incident reporting procedure, examples of which can be found in the HTA's Research Licensing Standards and Guidance document. This will help to remind staff about the types of incidents which will need to be reported and investigated internally.
4.	PFE2(c)	The DI should consider adopting regular trend analysis of critical storage temperatures. This may help with preventative maintenance of equipment and detect any malfunctioning equipment ahead of a possible failure.
5.	PFE2(c)	The DI should consider introducing regular alarm system testing to check that the alarm notification process is working as expected.

Background

Vertex Pharmaceuticals Limited (the establishment) has interests in several disease areas. The establishment stores and uses human tissue from living and deceased donors for research, from suppliers within and outside of the United Kingdom (UK). No consent is sought at the establishment.

This was the second routine inspection of the establishment since it was first granted a licence in 2012.

Description of inspection activities undertaken

The HTA's regulatory requirements are set out in Appendix 1. The Regulation Manager covered the following areas during the inspection:

Standards assessed against during inspection

Of the 47 HTA standards, 40 standards were assessed (standards published 3 April 2017). HTA standards, C1(b),(d),(e),(f) and C2(a),(b),(c) were not applicable as the establishment is not directly involved in seeking consent.

Review of governance documentation

A number of documents were reviewed during the assessment, which included but were not limited to, standard operating procedures for licensable activities, key policies, traceability audits, meeting minutes, staff training records, sample tracking system, temperature monitoring data and incidents.

Visual inspection

There was no visual inspection of the premises; however, a meeting took place with relevant staff members to discuss the PFE standards. Photographs of premises and storage areas were reviewed during the assessment.

Audit of records

No traceability audit was carried out; however, a review of recently conducted audits around HTA standards and traceability was undertaken as part of the assessment.

Meetings with establishment staff

A roundtable discussion was carried out with establishment staff which included the DI, Person Designated (PD) and researchers working under the licence.

Report sent to DI for factual accuracy: 24 June 2024

Report returned from DI: 15 July 2024 (no comments)

Final report issued: 16 July 2024

Completion of corrective and preventative actions (CAPA) plan

Based on information provided, the HTA is satisfied that the establishment has completed the agreed actions in the CAPA plan and in doing so has taken sufficient action to correct all shortfalls addressed in the Inspection Report.

Date: 1 November 2024

Appendix 1: The HTA's regulatory requirements

Prior to the grant of a licence, the HTA must assure itself that the DI is a suitable person to supervise the activity authorised by the licence and that the premises are suitable for the activity.

The statutory duties of the DI are set down in Section 18 of the Human Tissue Act 2004. They are to secure that:

- the other persons to whom the licence applies are suitable persons to participate in the carrying-on of the licensed activity;
- suitable practices are used in the course of carrying on that activity; and
- the conditions of the licence are complied with.

Its programme of inspections to assess compliance with HTA licensing standards is one of the assurance mechanisms used by the HTA.

The HTA developed its licensing standards with input from its stakeholders. They are designed to ensure the safe and ethical use of human tissue and the dignified and respectful treatment of the deceased. They are grouped under four headings:

- consent
- governance and quality systems
- traceability
- premises facilities and equipment.

This is an exception-based report: only those standards that have been assessed as not met are included. Where the HTA determines that there has been a shortfall against a standard, the level of the shortfall is classified as 'Critical', 'Major' or 'Minor' (see Appendix 2: Classification of the level of shortfall). Where HTA standards are fully met, but the HTA has identified an area of practice that could be further improved, advice is provided.

HTA inspection reports are published on the HTA's website.

Appendix 2: Classification of the level of shortfall

Where the HTA determines that a licensing standard is not met, the improvements required will be stated and the level of the shortfall will be classified as ‘Critical’, ‘Major’ or ‘Minor’. Where the HTA is not presented with evidence that an establishment meets the requirements of an expected standard, it works on the premise that a lack of evidence indicates a shortfall.

The action an establishment will be required to make following the identification of a shortfall is based on the HTA's assessment of risk of harm and/or a breach of the Human Tissue Act 2004 (HT Act) or associated Directions.

1. **Critical shortfall:**

   A shortfall which poses a significant risk to human safety and/or dignity or is a breach of the HT Act or associated Directions

   or

   A combination of several major shortfalls, none of which is critical on its own, but which together could constitute a critical shortfall and should be explained and reported as such.

   A critical shortfall may result in one or more of the following:

   - A notice of proposal being issued to revoke the licence
   - Some or all of the licensable activity at the establishment ceasing with immediate effect until a corrective action plan is developed, agreed by the HTA and implemented.
   - A notice of suspension of licensable activities
   - Additional conditions being proposed
   - Directions being issued requiring specific action to be taken straightaway

2. **Major shortfall:**

   A non-critical shortfall that:

   - poses a risk to human safety and/or dignity, or
   - indicates a failure to carry out satisfactory procedures, or
   - indicates a breach of the relevant Codes of Practice, the HT Act and other relevant professional and statutory guidelines, or

- has the potential to become a critical shortfall unless addressed

or

A combination of several minor shortfalls, none of which is major on its own, but which, together, could constitute a major shortfall and should be explained and reported as such.

In response to a major shortfall, an establishment is expected to implement corrective and preventative actions within 1-2 months of the issue of the final inspection report. Major shortfalls pose a higher level of risk and therefore a shorter deadline is given, compared to minor shortfalls, to ensure the level of risk is reduced in an appropriate timeframe.

3. **Minor shortfall:**

   A shortfall which cannot be classified as either critical or major, but which indicates a departure from expected standards.

   This category of shortfall requires the development of a corrective action plan, the results of which will usually be assessed by the HTA either by desk based review or at the time of the next inspection.

   In response to a minor shortfall, an establishment is expected to implement corrective and preventative actions within 3-4 months of the issue of the final inspection report.

Follow up actions

A template corrective and preventative action plan will be sent as a separate Word document with the final inspection report. Establishments must complete this template and return it to the HTA within 14 days of the issue of the final report.

Based on the level of the shortfall, the HTA will consider the most suitable type of follow-up of the completion of the corrective and preventative action plan. This may include a combination of

- a follow-up inspection
- a request for information that shows completion of actions
- monitoring of the action plan completion
- follow up at next routine inspection.

After an assessment of the proposed action plan establishments will be notified of the follow-up approach the HTA will take.