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Lysosomal Storage Disorder Charitable Access Program (LSD CAP)

Takeda

Submitted as part of Access Accelerated

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The information in this report has been submitted by the company concerned to the Access Observatory at Boston University. The information will be updated regularly. For more information about the Observatory go to www.accessobservatory.org

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Program Description

Program Overview

1 Program Name

Takeda Lysosomal Storage Disorder Charitable Access Program (LSD CAP)

2 Diseases program aims to address

- Other NCD: Lysosomal Storage Disorder

3 Beneficiary population

- Age Group: All ages
- Gender: All genders
- Special Populations: Low income, rural, urban, underserved communities

4 Countries

- India
- Egypt
- Pakistan
- Tunisia
- Paraguay
- Morocco
- Belarus
- Albania
- Jordan
- Bosnia and Herzegovina
- Tanzania
- Sudan

5 Program start date

January 1, 2013

6 Anticipated program completion date

Completion date not specified.

7 Contact person

Philip Towle (Philip.Towle@takeda.com)

8 Program summary

There are an estimated 350 million people living with rare diseases around the world¹, and they are often severe, life-threatening and complex life-long conditions.

Our Charitable Access Program (CAP), established in partnership with several non-governmental organizations, aims to improve access to therapies for patients with rare and genetic diseases regardless of where they may live. Through the CAP, we provide structured charitable donations of our products for patients with rare genetic conditions. We continue to explore options for charitable donations through strong partnerships with third-party organizations that help support patients with this disease, regardless of where they live and their ability to pay for treatment.

In addition to product donation, the LSD CAP provides support for local physicians with ongoing educational programs about rare diseases, diagnosis, and management to support long-term, locally sustainable patient care.

The key objective of this patient-centric program focuses on not only ensuring stable and continuous access to treatment for patients who might not otherwise have access to treatment, but also at the same time, supporting country capacity building for sustainable long-term patient care by understanding local challenges and unmet needs in underserved communities. Capacity building includes: supporting with regular Educational training, with focus on practical and sustained (digital based) knowledge building around disease awareness, clinical skills & management and diagnostic challenges.

How the LSD CAP works:

The CAP is currently available for patients with three of the most common lysosomal storage disorders (LSD), Fabry disease, Gaucher disease and Hunter syndrome. The program provides contributions of medical products to non-profits and non-governmental organizations in select countries where i) Takeda's products are not available; ii) some patients have been diagnosed; iii) no local regulatory filing is planned (or is more than 12-24 months away); and iv) in-country capabilities exist to safely and effectively administer treatments.

Takeda is the primary provider of LSD product donation, funding, educational medical resources and support. The program is delivered in partnership with expert not-for-profit organizations including Direct Relief and Project Hope, who collaborate with partners who have a shared mission to support patient access to innovative and often life-saving treatment. This includes patient association groups and governmental bodies. The programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.

Program Overview

8 Program summary cont.

Patient treatment and monitoring

Applications for participation in the LSD CAP are submitted by the treating physician. The application may also include a confidential short video of the patient's clinical state as appropriate, to allow reviewers to more accurately assess patient clinical severity and thus potential to benefit from treatment.

Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.

We also uphold patient adherence protocols and work with healthcare professionals to provide ongoing patient support to maximize the medical benefits of the drugs - including:

- 1) Attending the treatment center to receive their infusions every week or every other week (dependent on disease requirement)
- 2) Agreeing to regular physician follow-up visits and routine blood tests to allow for monitoring of response to treatment, Completing an annual quality-of-life survey.

Program Strategies & Activities

9 Strategies and activities

Strategy 1: Health Service Strengthening

ACTIVITY	DESCRIPTION
Planning	Planning with external parties (such as Patient Organisations, Health Authorities and physicians) where relevant to understand local country needs and challenges.
Training	<p>Disease education:</p> <p>Face to face regional training (Lysosomal Storage Disorder (LSD) and other rare metabolic diseases) followed by enrollment online program and consultation support executed through partner NGO.</p> <p>Targeted preceptorships (hands-on basic disease/management training) at leading global Centers of Excellence for physicians with enrolled patients.</p>
Management	<p>The program is delivered in partnership with expert not-for-profit organizations (namely Direct Relief and Project Hope), who collaborate with partners who have a shared mission to support patient access to innovative and often life-saving treatment. These include patient association groups and governmental bodies.</p> <p>In addition, the programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.</p> <p>Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.</p> <p>We also uphold patient adherence protocols and work with healthcare professionals to provide ongoing patient support to maximize the medical benefits of the drugs - including:</p> <ol style="list-style-type: none"> 1) Attending the treatment center to receive their infusions every week or every other week (dependent on disease requirement) 2) Agreeing to regular physician follow-up visits and routine blood tests to allow for monitoring of response to treatment, Completing an annual quality-of-life survey.
Funding	Grants support to local Patient Association Groups via Direct Relief (e.g. for patient/ caregiver travel to treatment centers).

Strategy 2: Medicine Donation

ACTIVITY	DESCRIPTION
Donation	Provide structured Charitable Donations of our products for patients with rare, genetic conditions such as Lysosomal Storage Disorder.

Companies, Partners & Stakeholders

10 Strategy by country

STRATEGY	COUNTRY
Medicine Donation	Egypt, Botswana, Ethiopia, Ghana, Indonesia, India, Kenya, Morocco, Pakistan, Paraguay, Sudan, Tanzania, West Bank and Gaza
Health Service Strengthening	[No response provided]

11 Company roles

COMPANY	ROLE
Takeda	Our commitment to patients includes improving access to therapies regardless of where patients may live. Takeda has delivered on this commitment by providing charitable donations of our products for patients with rare, genetic, hematologic, and immunologic conditions.

12 Funding and implementing partners

PARTNER	ROLE/URL	SECTOR
Direct Relief	The CAP program is centralized through not-for-profit logistics partners (Direct Relief and Project Hope). The Direct Relief managed program supports with the logistical supply of the donated therapy into the relevant countries as listed. www.directrelief.org	Voluntary
Project HOPE	Project Hope is an NGO that supports 12 patients with Gaucher Disease in Egypt with logistics support of the donated therapy into the country. www.projecthope.org	Voluntary

Companies, Partners & Stakeholders

13 Funding and implementing partners by country

PARTNER	COUNTRY
Project HOPE	Egypt
Direct Relief	Botswana, Ghana, Indonesia, India, Kenya, Morocco, Pakistan, Paraguay, Sudan, West Bank and Gaza, Egypt.

14 Stakeholders

STAKEHOLDER	DESCRIPTION OF ENGAGEMENT	REQUESTED OR RECEIVED
Non-government organization (NGO)	<p>The program is delivered in partnership with expert not-for-profit organizations including Direct Relief and Project Hope, who collaborate with partners who have a shared mission to support patient access to innovative and often life-saving treatment. This includes patient association groups and governmental bodies. The programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.</p> <p>http://www.wvi.org/</p>	<p>Infrastructure: No Human Resources: Yes Funding: yes Monitoring or Oversight: Yes Other resource: No</p>
Other	<p>The programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.</p>	[No response provided]

Local Context, Equity & Sustainability

15 Local health needs addressed by program

There are an estimated 350 million people living with rare diseases around the world¹, and they are often severe, life-threatening and complex conditions.

Our Charitable Access Program (CAP), established in partnership with several non-governmental organizations, aims to improve access to therapies for patients with rare and genetic diseases regardless of where they may live. Through the CAP, we provide structured charitable donations of our products for patients with rare genetic conditions. We continue to explore options for charitable donations through strong partnerships with third-party organizations that help support patients with this disease, regardless of where they live and their ability to pay for treatment. In addition to product donation, the LSD CAP provides support for local physicians with ongoing educational programs about rare diseases, diagnosis, and management to support long-term, locally sustainable patient care.

The key objective of this patient-centric program focuses on not only ensuring stable and continuous access to treatment for patients who might not otherwise have access to treatment, but also at the same time, supporting country capacity building for sustainable long-term patient care by understanding local challenges and unmet needs in underserved communities. Capacity building includes: supporting with regular Educational training, with focus on practical and sustained (digital based) knowledge building around disease awareness, clinical skills & management and diagnostic challenges.

a How needs were assessed

No formal needs assessment carried out.

Information obtained from extensive discussions with patient organisations and local country insight gained from first-hand country visits and/ or patient organisations experience or physician disease experts insights that worked in the respective countries on these diseases.

b Formal needs assessment conducted

No

16 Social inequity addressed

There are an estimated 350 million people living with rare diseases around the world (1), and they are often severe, life-threatening and complex conditions.

Our Charitable Access Program (CAP), established in partnership with several non-governmental organizations, aims to improve access to therapies for patients with rare and genetic diseases regardless of where they may live. Through the CAP, we provide structured charitable donations of our products for patients with rare genetic conditions. We continue to explore options for charitable donations through strong partnerships with third-party organizations that help support patients with this disease, regardless of where they live and their ability to pay for treatment.

In addition to product donation, the LSD CAP provides support for local physicians with ongoing educational programs about rare diseases, diagnosis, and management to support long-term, locally sustainable patient care.

The key objective of this patient-centric program focuses on not only ensuring stable and continuous access to treatment for patients who might not otherwise have access to treatment, but also at the same time, supporting country capacity building for sustainable long-term patient care by understanding local challenges and unmet needs in underserved communities. Capacity building includes: supporting with regular Educational training, with focus on practical and sustained (digital based) knowledge building around disease awareness, clinical skills & management and diagnostic challenges.

Local Context, Equity & Sustainability

17 Local policies, practices, and laws considered during program design

POLICY, PRACTICE, LAW	APPLICABLE TO PROGRAM	DESCRIPTION OF HOW IT WAS TAKEN INTO CONSIDERATION
National Regulations	Yes	Drugs are highly regulated and each country has specific rules for importation and distribution, in particular for donated product, so an NGO partner(s) with deep expertise in these aspects was selected.
Procurement procedures	[No response provided]	[No response provided]
Standard Treatment Guidelines	[No response provided]	[No response provided]
Quality and Safety Requirements	Yes	The donated drugs require cold chain supply so collaboration with logistics partners with deep expertise is critical.
Remuneration scales and hiring practices	[No response provided]	[No response provided]

18 How diversion of resources from other public health priorities are avoided

Public health authorities are not directly involved in this program.

19 Program provides health technologies (medical devices, medicines, and vaccines)

TYPE	COMMERCIAL NAME	INTERNATIONAL NON-PROPRIETARY NAME
Medicine	Elaprase	Idursalfase
Medicine	VPRIV	Velaglucerase alfa
Medicine	Replagal	Agalsidase alfa

Local Context, Equity & Sustainability

20 Health technology(ies) are part of local standard treatment guidelines

No. These drugs are not yet registered or approved in the local countries and regions where we donate the drug to. The drugs however do have global marketing authorization in at least one major global market.

21 Health technologies are covered by local health insurance schemes

No. The drugs we donate are not covered by local health insurance schemes.

22 Program provides medicines listed on the National Essential Medicines List

No.

23 Sustainability plan

Patient organisations are currently working globally advocating with United Nations to get rare diseases recognized as part of the universal right of health care. NGO Committee for Rare Diseases inaugurated by the UN is a global, multi-stakeholder initiative focusing on rare diseases. They aim to increase visibility of rare diseases at the global level, extend and share knowledge about rare diseases and their unmet needs, create a global platform to connect NGOs involved in rare diseases, promote international collaborations for rare diseases, and make rare diseases a global priority in public health, research and medical and social care policies. The program supports this by helping to identify and address some of the needs in countries that lack strong healthcare systems and supports local authorities in raising awareness for the need to prioritize rare diseases.

Additional Program Information

24 Additional program information

[No response provided]

a Potential conflict of interest discussed with government entity

No.

25 Access Accelerated Initiative participant

Yes.

26 International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) membership

Yes.

Resources

1. Global Genes. Global Genes Factsheet on Rare Diseases. <https://globalgenes.org/rare-diseases-facts-statistics/>

Program Indicators

PROGRAM NAME

Lysosomal Storage Disorder Charitable Access Program

27 List of indicator data to be reported into Access Observatory database

INDICATOR	TYPE	STRATEGY	2017	2018	2019	2020
1 Number of people trained	Output	Health Service Strengthening	---	---	113 people	---
2 Number of individuals receiving donated medicines	Output	Medicine Donation	---	---	228 people	---
3 Patients retained in care	Outcome	Health Service Strengthening	---	---	57:64 people	---
4 Number of patients on treatment	Outcome	Health Service Strengthening	---	---	----	---
5 Volume of medicines donated	Output	Medicine Donation	---	---	91,809 vials	---

INDICATOR **Number of people trained**

STRATEGY HEALTH SERVICE STRENGTHENING

ITEM	DESCRIPTION
Definition	Number of trainees
Method of measurement	Counting of people who completed all training requirements Calculation: Sum of the number of people trained
28 Data source	Routine program data
29 Frequency of reporting	Once per year

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
30 Data collection	Implementing partners: Project HOPE, Direct Relief.	Our implementing partners manage and record attendance, cadre, additional demographic details (such as gender, etc.) which are signed by participants.	One-time event
31 Data processing	Implementing partners: Project HOPE, Direct Relief.	Our implementing partners manage and record attendance, cadre, additional demographic details (such as gender, etc.) which are signed by participants.	Once per year
32 Data validation		A member of my company works closely with our implementing partner to consolidate and verify the data collection and management process.	

33 Challenges in data collection and steps to address challenges

None.

INDICATOR	2017	2018	2019	2020
1 Number of people trained	---	---	113 people	---

Comments: The data represented is collected by our implementing partners, Direct Relief and Project Hope. 113 Healthcare Professionals were trained as part of the LSD CAP.

INDICATOR **Number of individuals receiving donated medicines**

2

STRATEGY MEDICINE DONATION

ITEM	DESCRIPTION
Definition	Number of individuals receiving donated medicines
Method of measurement	Counting the patients who received the donated medicines Calculation: Sum of all patients who received the donated medicines
Data source	Routine program data
Frequency of reporting	Once per year

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
30 Data collection	Implementing partners: Project HOPE, Direct Relief.	Our partners keep a record of the number of individuals that are benefiting from Takeda's Lysosomal Storage Disorder Charitable Access Program (LSD CAP). Aggregated and anonymized data is provided to Takeda Pharmaceuticals. Takeda correlate data from various sources, including externally from distributors and internally from finance and supply systems, against our partners data to validate our reporting of number of individuals receiving donated medicines.	Ongoing
31 Data processing	Implementing partners: Project HOPE, Direct Relief.	Our partners keep a record of the number of individuals that are benefiting from Takeda's Lysosomal Storage Disorder Charitable Access Program (LSD CAP). This data is then reported to Takeda's Access to Medicines office. Aggregated and anonymized data is provided to Takeda Pharmaceuticals. Takeda correlate data from various sources, including externally from distributors and internally from finance and supply systems, against our partners data to validate our reporting of number of individuals receiving donated medicines.	Once per year
32 Data validation		An internal audit performed every two years, verifies data collection and management procedures.	

33 Challenges in data collection and steps to address challenges

[No response provided]

INDICATOR Number of individuals receiving donated medicines

STRATEGY MEDICINE DONATION

INDICATOR	2017	2018	2019	2020
2 Number of individuals receiving donated medicines	---	---	228 people	---

Comments: The data represented is collected by our implementing partners, Direct Relief and Project Hope. 228 represents the number of patients who are currently ACTIVE on Takeda's Lysosomal Storage Disorder Charitable Access Program (LSD CAP) as of 31 January 2020. The number of patients who are currently active on Takeda's LSD CAP as of 31 January 2020 is disaggregated by country as follows: Albania - 1 Belarus - 5 Bosnia - 1 Egypt - 33 India - 64 Jordan - 1 Kenya - 1 Morocco - 8 Pakistan - 52 Palestine - 1 Paraguay -16 South Africa - 2 Sudan -12 Tanzania - 6 Tunisia - 25 The number of patients who are currently active on Takeda's LSD CAP as of 31 January 2020 is further disaggregated by Rare Disease Therapeutic Area AND associated product as follows: (A) Rare Diseases Therapeutic Area: Genetic Diseases = 226 patients (i) GAUCHER patients who are receiving VPRIV® donations = 163 Belarus - 5 Bosnia - 1 Egypt - 22 India - 34 Jordan - 1 Morocco - 4 Pakistan - 45 Palestine - 1 Paraguay - 15 Sudan - 11 Tanzania - 6 Tunisia - 18 (ii) FABRY patients who are receiving REPLAGAL® donations = 15 Albania - 1 Egypt - 1 India - 5 Morocco - 4 Paraguay - 1 Tunisia - 3 (iii) HUNTER or MPS II patients who are receiving ELAPRASE® donations = 48 Egypt - 10 India - 25 Kenya - 1 Pakistan - 7 Sudan - 1 Tunisia - 4 (B) Rare Diseases Therapeutic Area: Immunology = 2 patients HEREDITARY ANGIOEDEMA (HAE) patients receiving FIRAZYR® donations as of 31 January 2020 South Africa - 2 The number of patients who were ENROLLED as of 31 January 2020 is 256, disaggregated by country as follows: Albania 2 Belarus 5 Bolivia 1 Bosnia 1 Egypt 38 India 73 Jordan 1 Kenya 1 Morocco 12 Pakistan 57 Palestine 1 Paraguay 16 South Africa 2 South Korea 1 Sudan 12 Tanzania 6 Tunisia 27

INDICATOR Patients retained in care

STRATEGY HEALTH SERVICE STRENGTHENING

ITEM	DESCRIPTION
Definition	Percentage of registered patients who had a facility visit out of total number of registered patients expected to receive treatment for a specific condition within that time period (e.g. month)
Method of measurement	The health facility patient registry should provide information on the number of patient registered with the health facility. Calculation: <u>Number of registered patients attending the point of care</u> Number of registered patients expected to attend within that time period
28 Data source	Routine program data, External Non-public data
29 Frequency of reporting	Once per year

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
30 Data collection	Implementing partners: Project HOPE, Direct Relief.	<p>Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.</p> <p>We also uphold patient adherence protocols and work with healthcare professionals to provide ongoing patient support to maximize the medical benefits of the drugs - including:</p> <ol style="list-style-type: none"> 1) Attending the treatment center to receive their infusions every week or every other week (dependent on disease requirement) 2) Agreeing to regular physician follow-up visits and routine blood tests to allow for monitoring of response to treatment, Completing an annual quality-of-life survey. <p>The implementing partner maintains a record of the individual patients with lysosomal storage disorder who are diagnosed and are registered in the program. The implementing partner also records and collates the number of patients who are expected to visit the facilities for care, and whether the visit occurred, or the patient was lost-to- follow-up.</p>	Ongoing

INDICATOR **Patients retained in care**

STRATEGY HEALTH SERVICE STRENGTHENING

<p>31 Data processing</p>	<p>Implementing partners: Project HOPE, Direct Relief.</p>	<p>Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.</p> <p>We also uphold patient adherence protocols and work with healthcare professionals to provide ongoing patient support to maximize the medical benefits of the drugs - including:</p> <ol style="list-style-type: none"> 1) Attending the treatment center to receive their infusions every week or every other week (dependent on disease requirement) 2) Agreeing to regular physician follow-up visits and routine blood tests to allow for monitoring of response to treatment, Completing an annual quality-of-life survey. <p>The implementing partner records the individual patients with lysosomal storage disorder who are diagnosed and included in the program. They also record and collate the number of patients who are expected to visit the facilities for care, and whether the visit occurred or the patient was lost-to-follow-up.</p>	<p>Ongoing</p>
<p>32 Data validation</p>		<p>An internal audit performed every two years, verifies data collection and management procedures.</p>	

33 Challenges in data collection and steps to address challenges

None.

INDICATOR	2017	2018	2019	2020
<p>3 Patients retained in care</p>	---	---	57:64 people	---

Comments: The data is collected by our implementing partners, Direct Relief and Project Hope, and represents the number of active patients as of 31 January 2020 / the number of patients who enrolled onto LSD CAP.

INDICATOR Number of patients on treatment

STRATEGY HEALTH SERVICE STRENGTHENING

ITEM	DESCRIPTION
Definition	Number of people that received treatment through the program
Method of measurement	Counting of people who received treatment through the program Calculation: Sum of the number of people treated
28 Data source	Routine program data
29 Frequency of reporting	Once per year

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
30 Data collection	Implementing partners: Project HOPE, Direct Relief.	<p>The program is delivered in partnership with expert not-for-profit organizations including Direct Relief and Project Hope, who collaborate with partners who have a shared mission to support patient access to innovative and often life-saving treatment. This includes Care Beyond Diagnosis, Centogene, patient association groups, and governmental bodies. The programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.</p> <p>Patient treatment and monitoring</p> <p>Applications for participation in the LSD CAP are submitted by the treating physician. The application may also include a confidential short video of the patient's clinical state as appropriate, to allow reviewers to more accurately assess patient clinical severity and thus potential to benefit from treatment.</p> <p>Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.</p>	Once per year

INDICATOR Number of patients on treatment

STRATEGY HEALTH SERVICE STRENGTHENING

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
31 Data processing	Implementing partners: Project HOPE, Direct Relief.	<p>The program is delivered in partnership with expert not-for-profit organizations including Direct Relief and Project Hope, who collaborate with partners who have a shared mission to support patient access to innovative and often life-saving treatment. This includes Care Beyond Diagnosis, Centogene, patient association groups, and governmental bodies. The programs are supported by an independent medical expert committee (MEC) consisting of leading LSD medical experts, representatives from rare disease patient association groups (International Gaucher Alliance, Fabry International Network, and MPS Europe), and an ethicist, all who volunteer their time.</p> <p>Patient treatment and monitoring</p> <p>Applications for participation in the LSD CAP are submitted by the treating physician. The application may also include a confidential short video of the patient's clinical state as appropriate, to allow reviewers to more accurately assess patient clinical severity and thus potential to benefit from treatment.</p> <p>Once a patient is accepted into the program, we focus on providing high quality of care for the patient - this includes ensuring our innovative drugs reach the patients efficiently and quickly, following all regulations and working with the treatment center and physicians.</p>	Once per year
32 Data validation		An internal audit of our implementing partner is performed every two years.	

33 Challenges in data collection and steps to address challenges

None.

INDICATOR	2017	2018	2019	2020
4 Number of patients on treatment	---	---	---	---

Comments: N/A.

INDICATOR Volume of medicines donated

STRATEGY MEDICINE DONATION

ITEM	DESCRIPTION
Definition	Number of units of donated medicines received by the intended recipient
Method of measurement	Count of number of units of donated medicines received by the intended recipient
28 Data source	Routine program data
29 Frequency of reporting	Once per year

	RESPONSIBLE PARTY	DESCRIPTION	FREQUENCY
30 Data collection	Implementing partners: Project HOPE, Direct Relief.	On a quarterly basis our partners provide data of the number of units of medicines donated across each of the countries. Aggregated and anonymized data is provided to Takeda Pharmaceuticals. Takeda correlate data from various sources, including externally from distributors and internally from finance and supply systems, against our partners data to validate our reporting of volume of medicines donated.	Ongoing
31 Data processing	Implementing partners: Project HOPE, Direct Relief.	On a quarterly basis our partners provide data of the number of units of medicines donated across each of the countries. Aggregated and anonymized data is provided to Takeda Pharmaceuticals. Takeda correlate data from various sources, including externally from distributors and internally from finance and supply systems, against our partners data to validate our reporting of volume of medicines donated.	Every three months
32 Data validation		An internal audit performed every two years, verifies data collection and management procedures.	

33 Challenges in data collection and steps to address challenges

None.

INDICATOR	2017	2018	2019	2020
5 Volume of medicines donated	---	---	91,809 vials	---

Comments: Takeda donated the following products in the LSD CAP as of 31 January 2020: VPRIV® donation - 69,015 vials. One vial contains 400 Units of velaglucerase alfa; total donation - 27,606,000 Units Elaprase® - 18,567 vials. One vial contains 6 mg of idursulfase; total donation - 111,402 mg Replagal® - 4,051 vials. One vial contains 3.5 mg of agalsidase alfa; total donation - 14,179 mg Firazyr® - 176 pre-filled syringes. One syringe contains 30 mg Icatibant; total donation - 5,280 mg.

Program Documents

Program Documents

1. Mehta, A., Ramaswami, U., Muenzer, J., et al. A Charitable Access Program for Under-served LSD Patients Worldwide. Poster presented at the 15th Annual WORLDSymposium, February 4–8, 2019; Orlando, Florida, USA. Available at: https://bit.ly/lsp_poster

Appendix

This program report is based on the information gathered from the Access Observatory questionnaire below.

Program Description

PROGRAM OVERVIEW

1 Program Name

2 Diseases program aims to address:

Please identify the disease(s) that your program aims to address (select all that apply).

3 Beneficiary population

Please identify the beneficiary population of this program (select all that apply).

4 Countries

Please select all countries that this program is being implemented in (select all that apply).

5 Program Start Date

6 Anticipated Program Completion Date

7 Contact person

On the public profile for this program, if you would like to display a contact person for this program, please list the name and email address here (i.e. someone from the public could email with questions about this program profile and data).

8 Program summary

Please provide a brief summary of your program including program objectives (e.g., the intended purposes and expected results of the program; if a pilot program, please note this). Please provide a URL, if available. Please limit replies to 750 words.

PROGRAM STRATEGIES & ACTIVITIES

9 Strategies and activities

Based on the BUSPH Taxonomy of Strategies, which strategy or strategies apply to your program (please select all that apply)?

10 Strategy by country

If you have registered one program for multiple countries, this question allows you to provide a bit more specificity about each country (e.g. some countries have different strategies, diseases, partners, etc.). Please complete these tables as applicable. For each portion you have selected from above (program strategies), please identify which country/countries these apply.

COMPANIES, PARTNERS AND STAKEHOLDERS

11 Company roles

Please identify all pharmaceutical companies, including yours, who are collaborating on this program:

What role does each company play in the implementation of your program?

12 Funding and implementing partners

Please identify all funding and implementing partners who are supporting the implementation of this program (Implementing partners is defined as either an associate government or non-government entity or agency that supplements the works of a larger organization or agency by helping to carry out institutional arrangements in line with the larger organization's goals and objectives.)

a. What role does each partner play in the implementation of your program? Please give background on the organization and describe the nature of the relationship between the organization and your company. Describe the local team's responsibilities for the program, with reference to the program strategies and activities. (response required for each partner selected).

b. For each partner, please categorize them as either a Public Sector, Private Sector, or Voluntary Sector partner.

(Public Sector is defined as government; Private Sector is defined as A business unit established, owned, and operated by private individuals for profit, instead of by or for any government or its agencies. Generation and return of profit to its owners or shareholders is emphasized; Voluntary Sector is defined as Organizations whose purpose is to benefit and enrich society, often without profit as a motive and with little or no government intervention. Unlike the private sector where the generation and return of profit to its owners is emphasized, money raised or earned by an organization in the voluntary sector is usually invested back into the community or the organization itself (ex. Charities, foundations, advocacy groups etc.))

c. Please provide the URL to the partner organizations' webpages

13 Funding and implementing partners by country

If you have registered one program for multiple countries, this question allows you to provide a bit more specificity about each country (e.g., some countries have different strategies, diseases, partners, etc.). Please complete these tables as applicable. For each portion you have selected from above (funding and implementing partners), please identify which country/countries these apply.

14 Stakeholders

Please describe how you have engaged with any of these local stakeholders in the planning and/or implementation of this program. (Stakeholders defined as individuals or entities who are involved in or affected by the execution or outcome of a project and may have influence and authority to dictate whether a project is a success or not (ex. Ministry of Health, NGO, Faith-based organization, etc.). Select all that apply.

- Government, please explain
- Non-Government Organization (NGO), please explain
- Faith-based organization, please explain
- Commercial sector, please explain
- Local hospitals/health facilities, please explain
- Local universities, please explain
- Other, please explain

LOCAL CONTEXT, EQUITY & SUSTAINABILITY

15 Local health needs addressed by program

Please describe how your program is responsive to local health needs and challenges (e.g., how you decided and worked together with local partners to determine that this program was appropriate for this context)?

a How were needs assessed

b Was a formal need assessment conducted

(Yes/No) If yes, please upload file or provide URL.

16 Social inequity addressed

Does your program aim to address social inequity in any way (if yes, please explain). (Inequity is defined as lack of fairness or justice. Sometime 'social disparities,' 'structural barriers' and 'oppression and discrimination' are used to describe the same phenomenon. In social sciences and public health social inequities refer to the systematic lack of fairness or justice related to gender, ethnicity, geographical location and religion. These unequal social relations and structures of power operate to produce experiences of inequitable health outcomes, treatment and access to care. Health and social programs are often designed with the aim to address the lack of fairness and adjust for these systematic failures of systems or policies.)*

*Reference: The definition was adapted from Ingram R et al. Social Inequities and Mental Health: A Scoping Review. Vancouver: Study for Gender Inequities and Mental Health, 2013.

17 Local policies, practices, and laws considered during program design

How have local policies, practices, and laws (e.g., infrastructure development regulations, education requirements, etc.) been taken into consideration when designing the program?

18 How diversion of resources from other public health priorities are avoided

Please explain how the program avoids diverting resources away from other public health priorities? (e.g. local human resources involved in program implementation diverted from other programs or activities).

19 Program provides health technologies

Does your program include health technologies (health technologies include medical devices, medicines, and vaccines developed to solve a health problem and improve quality of lives)? (Yes/No)

20 Health technology(ies) are part of local standard treatment guidelines

Are the health technology(ies) which are part of your program part of local standard treatment guidelines? (Yes/No) If not, what was the local need for these technologies?

21 Health technologies are covered by local health insurance schemes

Does your program include health technologies that are covered by local health insurance schemes? (Yes/No) If not, what are the local needs for these technologies?

22 Program provides medicines listed on the National Essential Medicines List

Does your program include medicines that are listed on the National Essential Medicines List? (Yes/No) If not, what was the local need for these technologies?

23 Sustainability plan

If applicable, please describe how you have planned for sustainability of the implementation of your program (ex. Creating a transition plan from your company to the local government during the development of the program).

ADDITIONAL PROGRAM INFORMATION

24 Additional program information

Is there any additional information that you would like to add about your program that has not been collected in other sections of the form?

a Potential conflict of interest discussed with government entity

Have you discussed with governmental entity potential conflicts of interest between the social aims of your program and your business activities? (Yes/No) If yes, please provide more details and the name of the government entity.

25 Access Accelerated Initiative participant

Is this program part of the Access Accelerated Initiative? (Yes/No)

26 International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) membership

Is your company a member of the International Federation of Pharmaceutical Manufacturers & Associations (IFPMA)? (Yes/No)

Program Indicators

INDICATOR DESCRIPTION

27 List of indicator data to be reported into Access Observatory database

For this program, activities, please select all inputs and impacts for which you plan to collect and report data into this database.

28 Data source

For this indicator, please select the data source(s) you will rely on.

29 Frequency of reporting

Indicate the frequency with which data for this indicator can be submitted to the Observatory.

30 Data collection

- Responsible party: For this indicator, please indicate the party/parties responsible for data collection.
- Data collection — Description: Please briefly describe the data source and collection procedure in detail.
- Data collection — Frequency: For this indicator, please indicate the frequency of data collection.

31 Data processing

- Responsible party: Please indicate all parties that conduct any processing of this data.
- Data processing— Description: Please briefly describe all processing procedures the data go through. Be explicit in describing the procedures, who enacts them, and the frequency of processing.
- Data processing — Frequency: What is the frequency with which this data is processed?

32 Data validation

Description: Describe the process (if any) your company uses to validate the quality of the data sent from the local team.

33 Challenges in data collection and steps to address challenges

Please indicate any challenges that you have in collecting data for this indicator and what you are doing to address those challenges.

