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Care for adults living with spinal muscular atrophy in Ukraine



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About the benchmarking report on SMA adult care



This short report complements the benchmarking report on SMA adult care published in 2024 to assess how care is provided for adults with spinal muscular atrophy (SMA) living in 22 European countries, as well as the OdySMA project, an SMA access atlas. The objective of this report is to provide an evidence-based resource for the SMA patient community to engage EU and national policymakers and increase awareness about the care challenges faced by adults living with SMA. It also features calls-to-action for policy modifications at both national and EU level, with a view to enhance care and improve the overall quality of life for adults living with SMA.

For more information
explore the SMA Adult
Care Benchmarking
Report

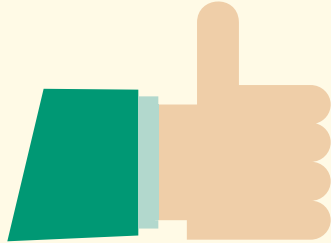


Visit OdySMA website
to find out more
about the project



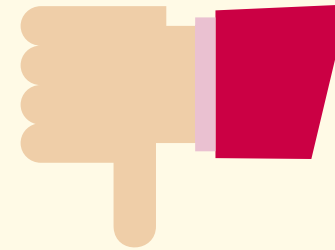
What it means to live as an adult with SMA in Ukraine

SOME GOOD NEWS



- There is a growing network of reference centres for the treatment of SMA in Ukraine.
- A Ministry of Health working group in Ukraine is developing a national SMA medical care protocol based on the international standards.
- Informal caregivers can potentially receive compensation as life assistants, even though obtaining reimbursement may be challenging.

SOME BAD NEWS



- Adults living with SMA have very limited national access to disease modifying treatments available across Europe, as well as to clinical trials.
- Although a registry from the Ukrainian Foundation for Children with SMA currently exists that collects data on adults living with SMA, it is not being used by the Ministry of Health.
- There is insufficient reimbursement for both medical and non-medical assistive devices, along with a general lack of support to enable adults living with SMA to live independently.



“

One of the organisation’s primary priorities is to facilitate access to treatment and rehabilitation for adults living with SMA. However, we can’t promise them either comprehensive treatment or appropriate equipment, because of the bureaucracy of the Ukrainian system.

**Representative from Ukrainian
Foundation for Children with SMA**

Overview of how Ukraine performed across the 19 indicators

Below is a summary of how Ukraine scored across all indicators in this benchmarking exercise.

For more information about the scoring system please consult the main report, namely Annex C.



● Good ● Reasonable ● Not good ● Poor ● No information available

Healthcare systems organisation



- 1 Transition from paediatric to adult care services
- 2 Navigation in the healthcare system in
- 3 Access to SMA treatment centres
- 4 Network of treatment centres
- 5 Patient registry
- 6 Standards of care (SoC) & treatment guidelines

Healthcare delivery



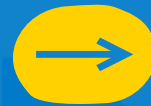
- 7 Multidisciplinary care
- 8 Shared decision-making
- 9 Continuity of care
- 10 Care team & caregivers training
- 11 Access to EMA-approved disease modifying therapies (DMTs)
- 12 Access to unauthorised disease modifying therapies (DMTs)

Governmental and peer support



- 13 HCP consultations reimbursement
- 14 Assistive equipment and devices support
- 15 Social, education and employment support
- 16 Life assistants/ professional caregivers
- 17 Informal caregivers
- 18 Rare disease policies and public funding for Patient Organisations
- 19 Patient Organisations' support

Deep dive into the 19 indicators and how
Ukraine compares with other countries



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Healthcare systems organisation

1. Transition from paediatric to adult care services

The Ukrainian Foundation for Children with SMA mentioned that there are no established procedures for transitioning individuals living with SMA from paediatric to adult care services. In fact, these services - including hospitals/healthcare centres and clinicians who oversee them - are entirely separate. [1] However, the majority of healthcare professionals (HCPs) responding to the survey (67%) mentioned that, in some cases, protocols or policies are deemed unnecessary because both children and adults living with SMA are treated by the same neurologists, so there is no need for a transition in care. [2] When transitioning from paediatric to adult care services, the majority of surveyed HCPs (67%) confirmed there is no need for re-eligibility for access to pharmacological treatment. [2]



2. Navigation in the healthcare system

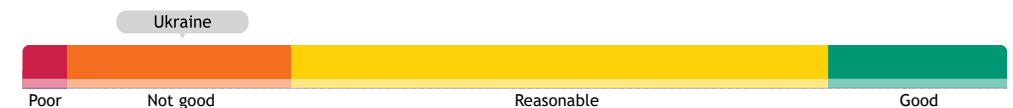
A few years ago, Ukraine initiated a family doctor system. Under this system, the doctor connects with the adult living with SMA and their family and, based on their needs, may refer him/her to different medical specialists. [1] In September 2022, the Ministry of Health issued an order for the establishment of centres of expertise, and a process was launched to review applications from healthcare centres. [3] In February/March 2023, a working session was organised, and a centre in Kharkiv was designated as the first expert centre for rare neuromuscular diseases (NMDs), with a specific focus on SMA and Duchenne muscular dystrophy. Two additional centres were established in the meantime in Lviv. [1] [4] No evidence could be found on the existence of care managers - contact persons who support adults living with SMA in organising interdisciplinary consultations,

taking over administrative/organisational tasks, and accessing government support measures - so the assumption is that this role does not exist in the Ukrainian healthcare system.



3. Access to SMA treatment centres

There are three expert centres on rare diseases, one located in Kharkiv (east), and two based in Lviv (west) that provide treatment for both children and adults living with SMA. [1] All HCPs who responded to the survey noted that there is an uneven distribution of specialised treatment centres for SMA or NMDs, and the majority (67%) stressed this also the case for general neurology clinics or departments. The even distribution is primarily seen in less specialised care, such as primary care. [2] Furthermore, transportation poses a challenge for adults living with SMA in Ukraine due to infrastructure gaps, such as railway systems not being wheelchair accessible. For those who do not reside in Kharkiv or Lviv, accessing the expert centres can be particularly challenging. [1] The majority of HCPs believe that fewer than half of adults receive care in the existing reference centres. As for obtaining an initial appointment with a specialist, adults living with SMA may have to wait less than a month, so waiting time is generally not an issue. [2]





Healthcare systems organisation

4. Network of treatment centres

The project titled “Pilot Network of Multidisciplinary Teams: A Case Study on SMA. The Path to Integration into the European Network of ERNs” began in the city of Kharkiv in 2018. The pilot project envisioned the establishment of a network of multidisciplinary teams with branches in Kyiv, Kharkiv, Lviv, Zaporizhzhia, and Odesa, with the possibility of including new members in the future. [5] [1] In the meantime, in September 2022, this project evolved, with the announcement of the establishment of a network of reference centres for rare (orphan) diseases in accordance with an Order of the Ministry of Health. [6] The majority of the HCPs who responded to the survey (67%) confirmed collaboration exists between adult SMA treatment centres, despite the fact that a formal network does not yet exist. [2] This might be because, at the time of the survey, the network was not yet operating.



5. Patient registry

The registry established in 2004 by the Ukrainian Foundation for Children with SMA is collecting data for both children and adults living with SMA in accordance with TREAT-NMD standards. [7] The Foundation is actively advocating for the Ministry of Health to incorporate this data into their policies. However, due to it being a patient-maintained registry owned by the Foundation, this is proving to be quite challenging. The Foundation's future plans include the integration of this registry within expert centres. These centres would utilise data from the Ukrainian patient-driven registry and acknowledge it as the official source of medical data. It is worth noting that another general state patient registry exists in Ukraine.

Unfortunately, the Ministry of Health is not able to, at this point, create additional registries. [1] When asked about the existence of a national registry collecting data on adults living with SMA, all HCPs who responded to the survey were also aware of its existence. [2]



6. Standards of care (SoC) & treatment guidelines

A working group within the Ministry of Health of Ukraine has been created and is working on a national protocol for SMA medical care, on the basis of the international standards of care. [3] [1] This document will regulate how care should be provided (including pharmacological treatment guidelines), not only in expert centres but also in any other healthcare centres, so that any patient can demand treatment according to this protocol, anywhere in Ukraine. This protocol will be finalised in 2023 for the Ministry of Health's adoption. [1] In addition, the Children with SMA Foundation and TREAT-NMD have also published the Ukrainian version of the “Guide to international standards of SMA treatment”. [8] There is limited evidence regarding the use of outcome measures, as the use is voluntary - since results do not need to be communicated for reimbursement. [1] Among the most used scales are the Revised Upper Limb Module (RULM), the Motor Function Measure (MFM-32), the Hammersmith Functional Motor Scale-Expanded (HFMSE) and the Revised Hammersmith Scale (RHS). [2] [1]





Healthcare delivery

7. Multidisciplinary care

According to the Children with SMA Foundation, currently, multidisciplinary teams (MDTs) are available only in the three expert centres located in Kharkiv (1) and Lviv (2). [1] All HCPs who responded to the survey had different points of view on the issue, with 33% saying that MDTs are not available, another 33% stating that different specialists are available but they don't meet formally as a MDT, and another 33% noting that MDTs are available and accessible. [2] This may mean that only a small percentage of the HCPs work in one of the three expert centres mentioned above and reflects the variability of care provision that exists beyond these expert centres in Ukraine.



8. Shared decision-making

Shared decision-making is an integral component of standard clinical practice. Nevertheless, certain challenges may arise, such as doctors encountering difficulties in engaging some adults living with SMA to actively participate. [2]



9. Continuity of care

Adults living with SMA are typically followed up 1-2 times per year (or more frequently), even if access to pharmacological treatment is not available. [1] According to the majority of HCPs who responded to the survey, there are no established processes to motivate adults living with SMA to stay engaged with the healthcare system. However, in certain instances, the family doctor might take on the role of a manager and assist the adult living with SMA in navigating the system. [2]



10. Care team & caregivers training

According to HCPs who responded to the survey, formal, in-person training for healthcare professionals is primarily conducted within the framework of expert centres. [2] Additionally, both HCPs and the Ukrainian Foundation for Children with SMA mentioned that pharmaceutical companies provide medical education. [1] [2] In fact, the Foundation confirmed that, thanks to training provided by pharmaceutical companies in previous years, they now have three physiotherapists trained in SMA care. [1] Training for individuals who provide social care services, often referred to as life assistants, is conducted by certified organisations following a standardised programme approved by the Ministry of Social Policy. This programme includes both theoretical and practical training, culminating in an examination. Once accepted into the official list, life assistants can choose the organisation from which they prefer to receive their training. [9] However, it's worth noting that there are currently no training opportunities or programmes available for informal caregivers who do not wish to enrol in the life assistant program.

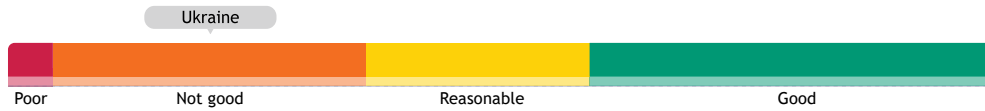




Healthcare delivery

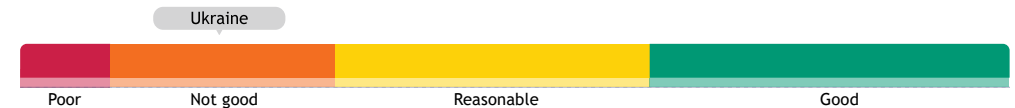
11. Access to EMA-approved disease modifying therapies (DMTs)

From a national perspective, adults living with SMA are unfortunately not eligible for the reimbursement of *nusinersen* (SPINRAZA®). [10] Regarding *risdiplam* (EVRYSDI®[▼]), while initially no adults living with SMA were eligible for reimbursement, criteria has been recently widened to adults living with SMA type 1 of any age, not on ongoing treatment with *nusinersen* and who require ventilatory support less than 16 hours per day. [11] Additionally local programs exist in some regions for *risdiplam* that cover adults living with SMA beyond this national criteria, including adults living with SMA type 2 and 3. [12] [13]



12. Access to unauthorised disease modifying therapies (DMTs)

No relevant pharma-sponsored clinical trials were identified over the the period of 2018-2022 for which adults living with SMA were eligible, including those listed on ClinicalTrials.gov and the European Union Clinical Trials Register. During the same period, an Early Access Programme was available for adults living with SMA for *risdiplam*. [1] [14]



Risdiplam (EVRYSDI®[▼]) has been approved by the European Medicines Agency (EMA) for the treatment of 5q spinal muscular atrophy (SMA) in patients with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies.
[▼]This compound is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via their national reporting system. See section 4.8 of the SmPC for details on how to report adverse reactions.



Governmental and peer support

13. HCP consultations reimbursement

The National Health Services of Ukraine reimburses medical care for all medical conditions through the 2023 Medical Guarantee Program, which includes 42 healthcare service packages (e.g., primary, secondary, and tertiary care, emergency medical care, medical rehabilitation, palliative care, and the State Reimbursement Program “Affordable Medicines”). [1] [15] Reimbursement is the same for all adults, whether they have SMA, rare diseases, or any other medical condition, as long as the healthcare services are included in the package. Recently, expert centres have been established, leading to the National Health Services considering an increase in the time for consultations for people living with rare diseases. In terms of specific medical specialties, while physiotherapy is fully reimbursed, it does not adequately address the needs of adults living with SMA due to a lack of training among physiotherapists. The same issue applies to scoliosis surgery, which is fully reimbursed but not commonly performed in Ukraine due to a shortage of specialists in this field. As a result, adults living with SMA often have to travel to Poland for such procedures. [1]



14. Assistive equipment and devices support

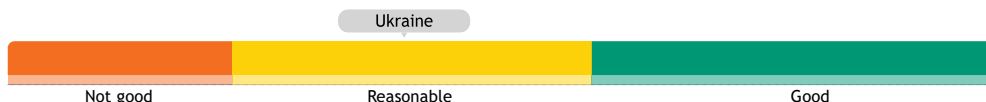
Medical equipment for individuals living with rare diseases are not covered by the tariff for medical services reimbursed under the Medical Guarantee Programme. [16] However, expenses with medical equipment may be covered by local programmes, mostly in bigger cities. [17] As per the Resolution of the Cabinet of Ministers of Ukraine dated 5 April, 2012, No. 321, persons with disabilities have the right to receive non-medical assistive devices free of charge through the Ministry of Social Policy. [18] However, equipping adults living with SMA for their daily needs remains a significant challenge. This is partly because the range of devices and equipment eligible for reimbursement is very limited, and only mechanical equipment, such as wheelchairs, strollers, and patient lifting devices, is covered, excluding home adaptation equipment (e.g., lifts to the second floor) or medical devices (e.g., ventilation

equipment, pulse oximeters). At the same time, the devices provided often do not meet the specific needs of these individuals. For instance, electric wheelchairs provided may not allow for the correct positioning of the back. [1] Looking at the workplace, enterprises, institutions, and organisations have the opportunity to create special workplaces for the employment of persons with disabilities, funded by the Fund for the Social Protection of Persons with Disabilities. [19] Even though this opportunity might, in theory, exist, the Ukrainian Foundation for Children with SMA pointed out it might not currently be working in practice, as they never witnessed fundamental changes being implemented. [1] Finally, there is no support provided to improve the accessibility of one’s residence or to facilitate relocation to a more accessible housing option from an infrastructure perspective. [1]



15. Social, education and employment support

Adults living with SMA are eligible for social benefits and assistance. The amount of these benefits and the determination of a care allowance depend on the disability subgroup they belong to and are governed by the “Law on State Social Assistance to Persons with Disabilities from Childhood and Children with Disabilities.” [20] However, it’s important to note that this support is often insufficient to meet the needs of adults living with SMA. [1] In addition to workplace modifications funded by the Fund for the Social Protection of Persons with Disabilities, persons with disabilities who cannot work on-site receive support from the state employment service to find employment that allows them to work from home. [19] Finally, the Ukrainian law grants persons with disabilities the right to attend either general or special education. [19] However, there do not appear to be specific provisions or programmes aimed at facilitating access to education for adults living with SMA.





Governmental and peer support

16. Life assistants/ professional caregivers

In October 2021, the Ukrainian government implemented a system to compensate individuals who offer professional social care services, including those provided to persons with disabilities. [1] [21] This compensation, regulated by the Law of Ukraine “On Social Services”, is calculated at 70% of the minimum hourly wage per hour per person, with a maximum limit of 360 hours per month. Adults living with SMA can take advantage of this system by submitting an application and choosing a life assistant from a list maintained by the relevant local authorities. [21]



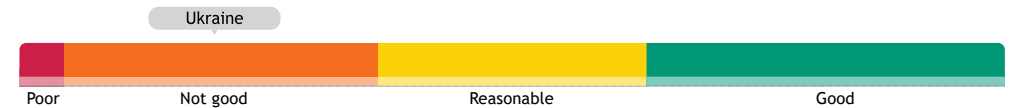
17. Informal caregivers

No financial or employment support is available for informal caregivers. However, according to Ukrainian Foundation Children with SMA, individuals who end up benefiting from the compensation on social care services provided on a professional basis to adults living with SMA are mostly parents or close relatives. This is because, under this position, their working hours count for the retirement pension. However, it is challenging to be appointed as life assistants and also receive the actual compensation. To help informal caregivers access this compensation, the Foundation provides learning courses. They have trained six individuals in 2023 who are now ready to apply to be a life assistant if they wish to proceed. [1]



18. Rare disease policies and public funding for Patient Organisations

In 2021, a national plan for rare diseases was adopted, covering the period of 2021 to 2026. This plan included various actions, one of which was the establishment of reference centres. [3] [22] As previously mentioned, in September 2022, the Ministry of Health initiated a process to evaluate applications from healthcare centres. As a result of this process, three expert centres for rare NMDs have been established, one in Kharkiv, and two in Lviv. [1] Additionally, the Ukrainian Foundation for Children with SMA does not receive any public funding to support its activities. [1]



19. Patient Organisations' support

The Ukrainian Foundation for Children with SMA was established in 2004 with a primary focus on children. At that time, there was limited awareness about adults living with SMA. One of the organisation's current key priorities is to provide adults living with SMA access to treatment and rehabilitation. Therefore, over the past few years, they have made significant efforts to connect with adults living with SMA. However, due to the lack of available treatment options, it is challenging to engage them in the community and daily activities. Many of them do not hold hope for improvement or a cure. Additionally, the organisation faces practical limitations within the bureaucratic Ukrainian system, making it a real challenge for these individuals' voices to be heard effectively. [1]



Conclusion



SMA Europe, with the endorsement of the Ukrainian Foundation for Children with SMA, calls on policymakers, healthcare systems, and the medical community to take coordinated action, in collaboration with the SMA patient community, to empower adults living with SMA in Ukraine to achieve meaningful health outcomes and quality of life as well as to have an independent and fulfilling life. We invite key stakeholders to, among other actions:

Further strengthen and leverage the registry established by the Ukrainian Foundation for Children with SMA, including by integrating it within the network of SMA/NMD adult treatment centres and ensuring that patient-relevant data, such as treatment outcomes, clinical history and natural history, are collected.

Ensure the network of SMA/NMD adult treatment centres facilitates SMA training programs for HCPs, including physiotherapists, to ensure multidisciplinary care teams within those, and HCPs outside of those, deliver care on the basis of best practices for adults living with SMA.

Address current inequalities in access to pharmacological treatment, which would include eliminating any existing barriers restricting adults' access to available disease modifying therapies, such as restricting eligibility for treatment to certain SMA types or ages.

Bibliography

- [1] *Ukrainian Foundation for Children with SMA*. [Interview]. July 2023.
- [2] *Online structured survey carried out with healthcare professionals treating adults living with SMA*, 11 Jan to 24 Mar, 2023.
- [3] Ministry of Health of Ukraine, "About the network of reference centers for rare (orphan) diseases," 7 September 2022. [Online]. Available: <https://zakon.rada.gov.ua/laws/show/z1119-22#Text>. [Accessed January 2023].
- [4] Ministry of Health of Ukraine, "List of reference centers for rare (orphan) diseases," 11 October 2023. [Online]. Available: <https://zakon.rada.gov.ua/rada/show/v0598282-23#Text>. [Accessed December 2023].
- [5] "Round table on the topic "Pilot Network of Multidisciplinary Teams on the example of SMA"," 2019.
- [6] Ministry of Health of Ukraine, "About the network of reference centers for rare (orphan) diseases," 7 September 2022. [Online]. Available: <https://zakon.rada.gov.ua/laws/show/z1119-22#Text>. [Accessed December 2023].
- [7] Ukrainian Foundation for Children with SMA, "A universal registration platform".
- [8] A. Shatillo, "Implementation of international recommendations for the management of patients with spinal muscular atrophy in clinical practice," *health-ua*, pp. 5-7, 21 10 2021.
- [9] Cabinet of Ministers of Ukraine, "Procedure for the training and retraining of natural persons who provide social care services without carrying out entrepreneurial activities," 1 Junho 2020. [Online]. Available: <https://zakon.rada.gov.ua/laws/show/430-2020-%D0%BF#n7>. [Accessed January 2023].
- [10] SMA Europe, "OdySMA project," 2023. [Online]. Available: <https://odysma.sma-europe.eu/countries/ukraine>. [Accessed 7 August 2023].
- [11] Ministry of Health of Ukraine, "METHODICAL RECOMMENDATIONS: planning and calculating the need for medicines for patients suffering from spinal muscular atrophy," 25 January 2023. [Online]. Available: <https://zakon.rada.gov.ua/rada/show/v0132282-23#Text>. [Accessed December 2023].
- [12] Lviv City Council, "Children from SMA will be provided with medicine for this year: a memorandum was signed in the Lviv City Hall," 18 October 2023. [Online]. Available: <https://city-adm.lviv.ua/news/science-and-health/medicine/298803-ditei-zi-sma-zabezpechat-likamy-na-tsei-rik-v-merii-lvova-pidpysaly-memorandum>. [Accessed December 2023].
- [13] Department of Health Protection, Executive Body of Kyiv City Council, "Provision of medicines for patients with spinal muscular atrophy (10.5.1.2.) - Comparative table for the project of changes to the city target program "Health of Kyivans" for 2020-2023," 4 November 2022. [Online]. Available: https://kmr.gov.ua/sites/default/files/1_zminy_programa_nova_versiya_04.11.22.xlsx?__cf_chl_tk=flyH7uhFdUsGsNH9pJqhtmUoQJLtgTuScRLCdXdrYk-1702656941-0-gaNycGzNDIA. [Accessed December 2023].
- [14] Roche Ukraine LLC, "Olga's story demonstrates that, despite the difficult diagnosis of SMA type I, thanks to treatment and joint efforts, one can continue to work, create and dream," November 2023. [Online]. Available: <https://sma.neuro-roche.com.ua/news/4102-storya-olgi-demonstru-sho-nezvazhayuchi-na-vazhkij-dagnoz-sma--tipu-zavdyak>. [Accessed December 2023].
- [15] National Health Service of Ukraine, "Medical Guarantee Program 2023 Requirements," [Online]. Available: <https://contracting.nszu.gov.ua/kontraktuvannya/kontraktuvannya-2023/vimogi-pmg-2023>. [Accessed January 2023].
- [16] N. H. S. o. Ukraine, "Orphan diseases: what free services can a patient receive under the Medical Guarantee Program," 09 04 2021. [Online]. Available: <https://nszu.gov.ua/novini/orfanni-hvorobi-yaki-bezoplatni-poslugi-mozhe-otrimati-paciy-527>.
- [17] Health Executive Committee of Zaporizhzhia City Council, "Program "Health care of the city of Zaporizhzhia for the period 2019-2021"," 29 January 2019. [Online]. Available: https://zp.gov.ua/upload/content/o_1d45gjjg949o91cd91n7mpbpjg91o.pdf. [Accessed December 2023].
- [18] Ministry of Social Policy of Ukraine, "Provision of technical and other means of rehabilitation," 5 April 2012. [Online]. Available: <https://www.msp.gov.ua/content/zabezpechennya-tehnichnimi-ta-inshimi-zasobami-reabilitacii-specavtotransportom.html>. [Accessed January 2023].
- [19] T. P. o. Ukraine, "The law of Ukraine. About the basics of social protection of persons with disabilities in Ukraine," [Online]. Available: <https://zakon.rada.gov.ua/laws/show/875-12#Text>.
- [20] M. Lazebna, "Assistance to families with children suffering from spinal muscular atrophy will be increased in Ukraine," Interfax, 21 12 2020. [Online]. Available: <https://ua.interfax.com.ua/news/interview/711390.html>. [Accessed January 2023].
- [21] Cabinet of Ministers of Ukraine, "Some issues of appointment and payment of compensation to natural persons who provide social care services without carrying out business activities on a professional basis," 6 October 2021. [Online]. Available: <https://zakon.rada.gov.ua/laws/show/1040-2021-%D0%BF#Text>. [Accessed January 2023].
- [22] Cabinet of Ministers of Ukraine, "Concept for the development of the system of providing medical care to patients suffering from rare (orphan) diseases for 2021-2026," 28 April 2021. [Online]. Available: <https://zakon.rada.gov.ua/laws/show/377-2021-%D1%80#Text>. [Accessed January 2023].

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