Designated Project "Evaluation and Application of Cost-Effectiveness of Pharmaceuticals and Medical Devices: Current Status and Future Prospects in Japan

"Responses from Pharmaceutical and Medical Device Companies"

October 29, 2021

Healthcare System Planning, Inc.
American Medical Device and IVD Manufacturers Association (AMDD), Institute for Medical Technology Policy
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HTA Target product (Excluding products that have been analyzed) : as of Oct 13, 2021

No.	総会指定	品目名	効能・効果	収載時価格※1	うち有用性系加算率	市場規模（ピーク時予測）	費用対効果評価区分	総会での指定日	現状
1	7	ノクサフィル※3（MSD）	深在性真菌症	3,109.10円（100mg錠）	なし	112億円	H１（市場規模が100億円以上）	2020/4/8	公的分析中
2	8	カボメティクス（武田薬品工業）	腎細胞癌、肝細胞癌※5	8,007.60円（20mg錠） 22,333.00円（60mg錠）	10%	127億円	H１（市場規模が100億円以上）	2020/5/13	公的分析中※5
3	9	エンハーツ（第一三共）	乳癌、胃癌※4	165,074円	5%	129億円	H１（市場規模が100億円以上）	2020/5/13	公的分析中※4
4	10	ゾルゲンスマ（ﾉﾊﾞﾙﾃｨｽﾌｧｰﾏ）	脊髄性筋萎縮症	167,077,222円	50%	42億円	H３（単価が高い）	2020/5/13	公的分析中
5	13	リベルサス（ﾉﾎﾞ ﾉﾙﾃﾞｨｽｸ ﾌｧｰﾏ）	２型糖尿病	143.20円（３mg錠） 334.20円（７mg錠） 501.30円（14mg錠）	５%	116億円	H１（市場規模が100億円以上）	2020/11/11	公的分析中
6	15	エムガルディ（日本ｲｰﾗｲﾘﾘｰ）	片頭痛	45,165円（120mg 1mL 1キット） 44,940円（120mg 1mL 1筒）	なし	173億円	H１（市場規模が100億円以上）	2021/4/14	企業分析中
7	17	ポライビー（中外製薬）	リンパ腫	298,825円（30mg） 1,364,330円（140mg）	５%	120億円	H１（市場規模が100億円以上）	2021/5/12	企業分析中
8	18	ダラキューロ（ﾔﾝｾﾝﾌｧｰﾏ）	多発性骨髄腫、全身性ALアミロイドーシス※6	432,209円	５%	370億円	H１（市場規模が100億円以上）	2021/5/12	企業分析中※6
9	19	アリケイス（ｲﾝｽﾒｯﾄﾞ）	肺非結核性抗酸菌症	42,408.40円	10%×0.2※2（２%）	177億円	H１（市場規模が100億円以上）	2021/5/12	企業分析中
10	22	アジョビ（大塚製薬）	片頭痛	41,356円（225mg1.5mL１筒）	なし	137億円	H５（エムガルディの類似品目）	2021/8/4	分析は行わない（エムガルディの評価に準じる）
11	23	アイモビーグ（ｱﾑｼﾞｪﾝ）	片頭痛	41,356円（70mg 1mL 1キット）	なし	153億円	H５（エムガルディの類似品目）	2021/8/4	分析は行わない（エムガルディの評価に準じる）
12	24	レベスティブ（武田薬品工業）	短腸症候群	79,302円（3.8mg１瓶）	５%	60億円	H２（市場規模が50億円以上）	2021/8/4	分析前協議中
13	25	ベクルリー（ｷﾞﾘｱﾄﾞ-ｻｲｴﾝｼｽﾞ）	SARS-CoV-2による感染症	63,342円（100mg１瓶）	なし	181億円	H１（市場規模が100億円以上）	2021/8/4	分析前協議中
14	26	Micra　経力テーテルペーシングシステム（日本ﾒﾄﾞﾄﾛﾆｯｸ）	※７	1,170,000円	10%	77億円	H２（市場規模が50億円以上）	2021/10/13	分析前協議中

※1　収載時価格は、キット特徴部分の原材料費除いた金額。
※2　加算係数（薬品総原価の開示度に応じた加算率）・・・開示度80%以上：1.0、50~80%：0.6、50%未満：0.2
※3　ノクサフィルは内用薬（ノクサフィル錠100mg）のみが費用対効果評価対象。
※4　胃癌については令和２年９月25日に効能追加され、公的分析中。
※5　肝細胞癌については令和２年11月27日に効能追加され、公的分析中。
※6　全身性ALアミロイドーシスについては令和３年８月25日に効能追加され、分析前協議中。
※7　主な使用目的：本品は、カテーテルを用いて経皮的に右心室内に留置される電極一体型の植込み型心臓ペースメーカである。なお、本品は撮像可能条件に適合する場合にのみ限定的にMRI検査が可能となる機器である。

HTA Target product (Products for which analysis has been completed) : as of Aug 4, 2021

No.	総会指定	品目名	効能・効果	市場規模（ピーク時予測）	費用対効果評価区分	総会での指定日	調整前価格	調整後価格	適用日
1	1	テリルジー100エリプタ（グラクソ・スミスクライン）	COPD（慢性閉塞性肺疾患）	236億円	H１（市場規模が100億円以上）	2019/5/15	4,183.50円（14吸入１キット） 8,853.80円（30吸入１キット）	4,160.80円（14吸入１キット） 8,805.10円（30吸入１キット）	2021/7/1
2	2	キムリア（ノバルティスファーマ）	白血病	72億円	H３（単価が高い）	2019/5/15	34,113,655円	32,647,761円	2021/7/1
3	3	ユルトミリス（アレクシオンファーマ）	発作性夜間ヘモグロビン尿症	331億円	H１（市場規模が100億円以上）	2019/8/28	730,894円	699,570円	2021/8/1
4	4	ビレーズトリエアロスフィア（アストラゼネカ）	COPD（慢性閉塞性肺疾患）	189億円	H５（テリルジーの類似品目）	2019/8/28	4,150.30円	4,127.60円	2021/7/1
5	12	エナジア（ノバルティスファーマ）	気管支喘息	251億円	H５（テリルジーの類似品目）	2020/8/19	291.90円（中用量） 333.40円（高用量）	290.30円（中用量） 331.50円（高用量）	2021/7/1
6	14	テリルジー200エリプタ（グラクソ・スミスクライン）	COPD（慢性閉塞性肺疾患）	130億円	H５（テリルジーの類似品目）	2021/2/10	4,764.50円（14吸入１キット） 10,098.90円（30吸入１キット）	4,738.50円（14吸入１キット） 10,043.30円（30吸入１キット）	2021/7/1
7	16	イエスカルタ（第一三共）	リンパ腫	79億円	H５（キムリアの類似品目）	2021/4/14	34,113,655円	32,647,761円	2021/4/21
8	20	ブレヤンジ（セルジーン）	リンパ腫	82億円	H５（キムリアの類似品目）	2021/5/12	34,113,655円	32,647,761円	2021/5/19
9	5	トリンテリックス（武田薬品工業）	うつ病・うつ状態	227億円	H１（市場規模が100億円以上）	2019/11/13	168.90円（10mg錠） 253.40円（20mg錠）	161.70円（10mg錠） 242.50円（20mg錠）	2021/11/1
10	6	コララン（小野薬品工業）	慢性心不全	57.5億円	H２（市場規模が50億円以上）	2019/11/13	82.90円（2.5mg錠） 145.40円（5mg錠） 201.90円（7.5mg錠）	変更なし	－
11	11	エンレスト（ノバルティスファーマ）	慢性心不全	141億円	H５（コラランの類似品目）	2020/8/19	65.70円（50mg錠） 115.20円（100mg錠） 201.90円（200mg錠）	変更なし	－
12	21	ベリキューボ（バイエル薬品）	慢性心不全	95億円	H５（エンレストの類似品目）	2021/8/4	131.50円（2.5mg錠） 230.40円（５mg錠） 403.80円（10mg錠）	変更なし	2021/8/12

Some thoughts on the process
In comparison with regulatory applications and insurance coverage applications

- Speed of response by the company
  - ✓In regulatory affairs and insurance, companies want a short process, whereas in cost-effectiveness assessment, there is no incentive to be early about the duration.
  - ✓Even in the pharmaceutical and insurance fields, once the dates for subcommittees and professional organizations are set, they are sometimes required to respond at an unreasonably fast pace, and I wonder if there is much difference.

- Decision-makers/involved parties
  - ✓In pharmaceutical affairs and insurance, clinical experts and other experts are involved in discussions and decisions in a wide range of ways, but it is common for the government to effectively make decisions before decision-making bodies such as subcommittees and professional organizations.
  - ✓In cost-effectiveness assessments, the direction of discussion is left to experts rather than to the government in many aspects, including pre-analysis consultations that have a major impact on the overall analysis.

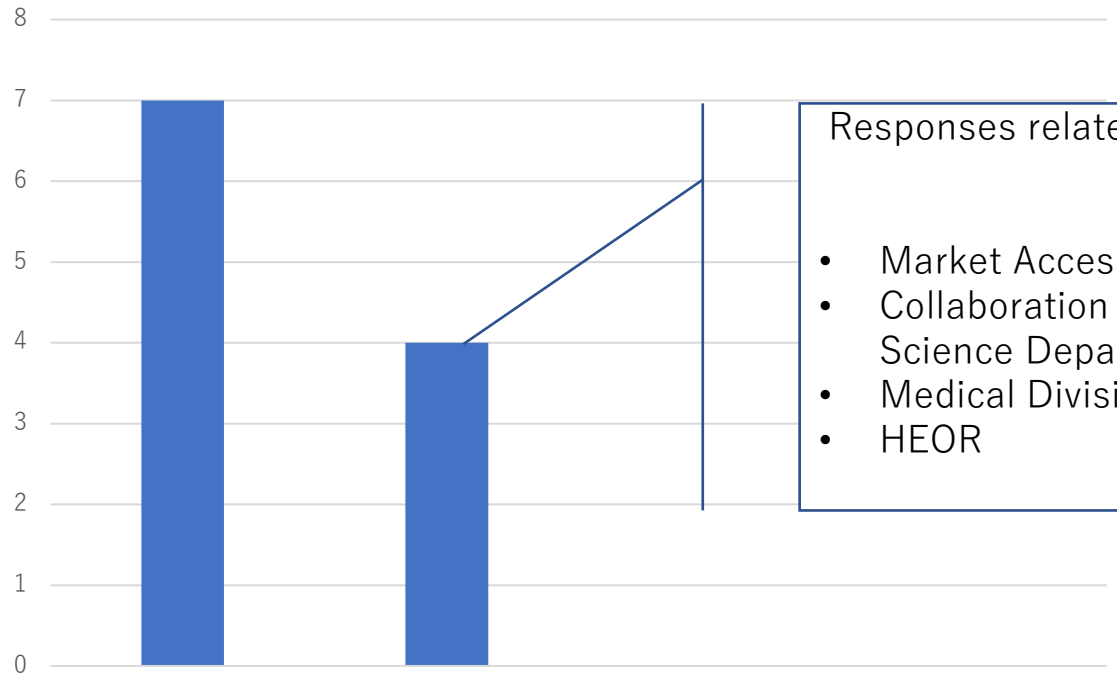
Results of a survey to the supporting members of ISPOR Japan Section

Outline of the survey

- Survey period: September 27 - October 2021 8 days
- Survey Target: 11 out of 26 ISPOR Japan Section supporting member companies (10 pharmaceutical companies, 1 medical device company)
- Questions
  I. Org structure related to HTA at your company
  II. Human resources needed for HTA
    1. Qualities needed for the person in charge
      a. Education (degrees, knowledge, etc.)
      b. experience
      c. ability
      d. Other
    2. Expectations for Academia to train HTA personnel
      a. Education for corporate personnel
      b. Other (e.g., students and other future personnel)
  III. Difficulties in establishing a system and taking action as a company to do HTA (including expectations of academia and government to resolve difficulties and concerns)

2. System at your company

Does your company have a department that specializes in cost-benefit assessment (HTA) systems?



Responses related to "existing departments that can respond as needed

- Market Access Department
- Collaboration in the Medical Affairs Department and Data Science Department
- Medical Division
- HEOR

3. Required human resources

(1) What qualities do you think are required of the person in charge of the HTA system at your company?

Education (degrees, knowledge, etc.)

- Expertise in health economics (scientific expertise and knowledge of the policy context in Japan)
- Knowledge of the target disease area, knowledge of clinical epidemiology
- Understanding of the basics and trends of the Japanese healthcare system, and knowledge of relevant overseas systems
- Master's level or higher in epidemiology, health economics, etc. preferred (but not required, as this will be handled by a
- team)
- Degree: MPH or PhD (medical/pharmacy/science and math are also acceptable, but require additional education)

Experience

- Experience in Japan's HTA system, from pre-analytical consultation to comprehensive evaluation and price adjustment, is desirable.
- 5+ years of experience in the pharmaceutical industry, preferably in health economics, biostatistics, regulatory affairs, market access, or project management related work
- Experience in promoting a series of project management activities from planning and implementation of research in HEOR/CEA related areas to the creation of outputs such as papers and conference presentations
- About 3 years in the clinical development or safety department of a pharmaceutical company

3. Required human resources

(1) What qualities do you think are required of the person in charge of the HTA system at your company?

ability

- Skills in areas related to cost-benefit analysis, high level of internal and external communication and negotiation skills, and project management skills
- Vendor Management
- English (understanding of papers, communication with global members)
- Strategic Mindset
- Scientific Background

Other

- Understanding the NHI drug pricing system
- It is important to recognize the need for a thorough understanding of the natural history, diagnosis, and treatment of the target disease, rather than a mechanical analysis.
- Logical thinking to prepare convincing government submissions and agility to respond to unplanned and unexpected events

3. Required human resources

(2) What do you expect from academia in order to train people in charge of the HTA system?

Education for corporate personnel

- I would like to see academia provide a number of educational programs in which companies can participate (e.g., Keio University's Health Economics Evaluation Human Resource Development Program, the University of Tokyo's HTA Expert Training Course).
- In the case of a global company, it is necessary to communicate with global staff in various areas such as modeling.
- Education from the perspective of medical economics and other specialties that are not too bound by the current cost-effectiveness evaluation system
- Acquire the skills necessary to evaluate the cost-effectiveness of the current situation (at a level that can be negotiated with C2H)
- Expect to be provided with opportunities to exchange opinions with the authorities.

Other

- I would like you to educate yourself on what HTA is all about.
- In addition to the academic point of view, we need to educate our students to acquire a business sense so that they can be immediately effective.
- Training of data scientists who can analyze the receipt database (medical database)
- I hope that you will promote education with a strict curriculum. In particular, I would like to see a doctoral program for those who will be in charge of authorities.
- I would like to see more people in academia who can consult with companies about what they are preparing.

Difficulties for companies in developing and implementing systems

- No matter how great the analysis is, it is difficult for HTA staff to stay motivated because they do not want a good evaluation when the results are downgraded.
- In many cases, the construction of disease models seems to be somewhat forced. I feel that if cost-effectiveness analysis is not conducted using appropriate analytical models, decisions based on cost-effectiveness analysis or the significance of conducting cost-effectiveness analysis in the first place will be undermined in the future. In order to resolve this issue, epidemiological data must be enriched so that an appropriate analysis model can be firmly established.

- When educating human resources, we would like them to be accurately educated on the differences between academic cost-effectiveness analysis and the cost-effectiveness evaluation system used for price adjustment.
- Under the current cost-effectiveness evaluation system, the person in charge of HTA in a company may be in a position where it is difficult to explain from the perspective of science, and it may be difficult to maintain motivation, as well as to establish a system and develop human resources in the company in the future. I would like to ask academicians to communicate that the HTA system should be operated in consideration of the impact on the development of corporate personnel and, in the long term, on the overall human resources involved in HTA.

Discussion of the Survey Results - In Lieu of a Summary

- With the cooperation of various parties, I believe that the corporate structure is being developed.
  - ➢ However, it takes time to develop human resources.
  - ➢ On the other hand, the disparity between companies (and industries) appears to be large.

- The situation of HTA staffs of manufactures in a difficult position came to light.
  - ➢ Where is the problem (Japanese system vs. foreign system, academia vs. reality, explanation technics)?
    - ✓ Is the background that HTA is done after the redemption price is determined and used for price adjustment?
  - ➢ Can we play a role other than restraining price reductions (e.g., relaxation of market expansion recalculation, hand-up method)?

- More learning opportunities for manufacturers

thanks

- I would like to express my sincere gratitude to everyone who cooperated in the survey for ISPOR members, and to Mr. Higashi, Director, and Mr. Kobayashi, Secretary General of ISPOR Japan Section, for their efforts in designing and implementing the survey.