Programa de Acesso Precoce e de Partilha de Risco em Oncologia

Ricardo da Luz

Centro Hospitalar Lisboa Central

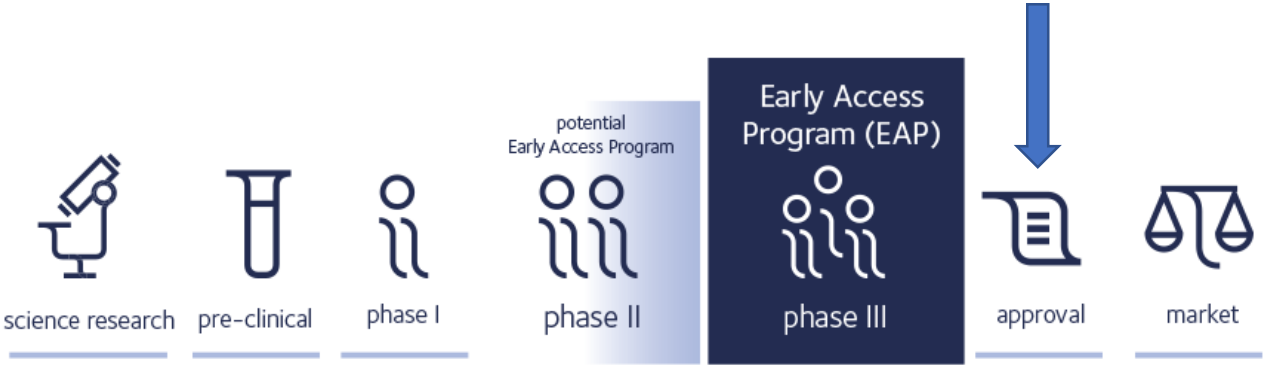
Programas de Acesso Precoce

Diário de Notícias – 3 de Dezembro 20
“500 doentes em estado grave ajudados com medicamentos sem aprovação do Infarmed”

Neoplasias – 384
Pulmão – 242
Melanoma – 82
Mieloma - 30
LLC – 30

Programas de Acesso Precoce

Desenvolvimento de um fármaco ≈ 15 anos



Formas de acesso precoces (tratamento antes da AIM):

- Ensaios Clínicos
- Programas de Acesso Expandido (compreendido, como ensaios clínicos com um objectivo de segurança)
- Programas de uso compassivo
- Pedidos Nominais

Programas de Acesso Precoce

Prós

- Utilização de medicamento nos doentes que necessitam, antes da comparticipação
- Sem encargos directos
- Aquisição de experiência clínica

Contras

- Alguns doentes apenas
- Nº de doentes limitado
- Prazo relativamente curto
- A divulgação não é equitativa
- Sem aquisição de conhecimento
- Utilização de fármacos sem informação completa

Programas de Acesso Precoce

Opdivo

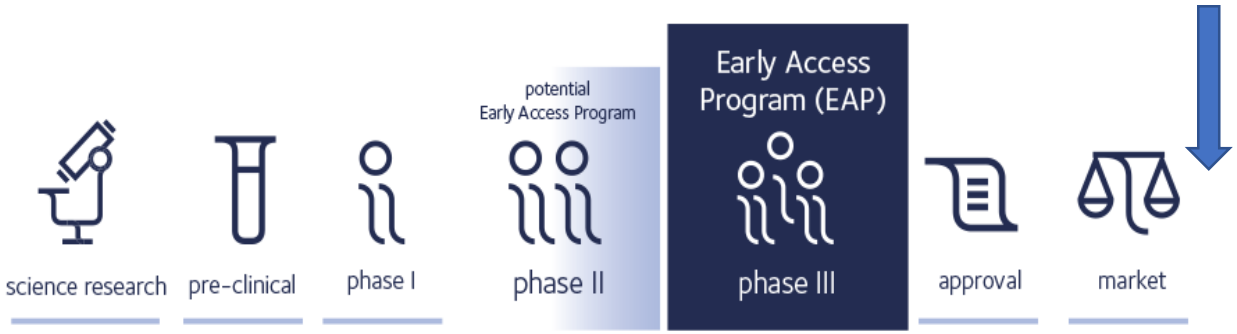
- 6 doentes
- Durou 3 meses desde que fomos informados até à sua conclusão
- Ainda não há comparticipação
- Ainda há doentes a fazer a medicação no EAP

Lonsurf

- 5 doentes
- Durou 1 mês desde que abriu para o nosso Centro
- Todos os doentes interromperam

Programas de Acesso Precoce

Desenvolvimento de um fármaco ≈ 15 anos



Partilha de Risco

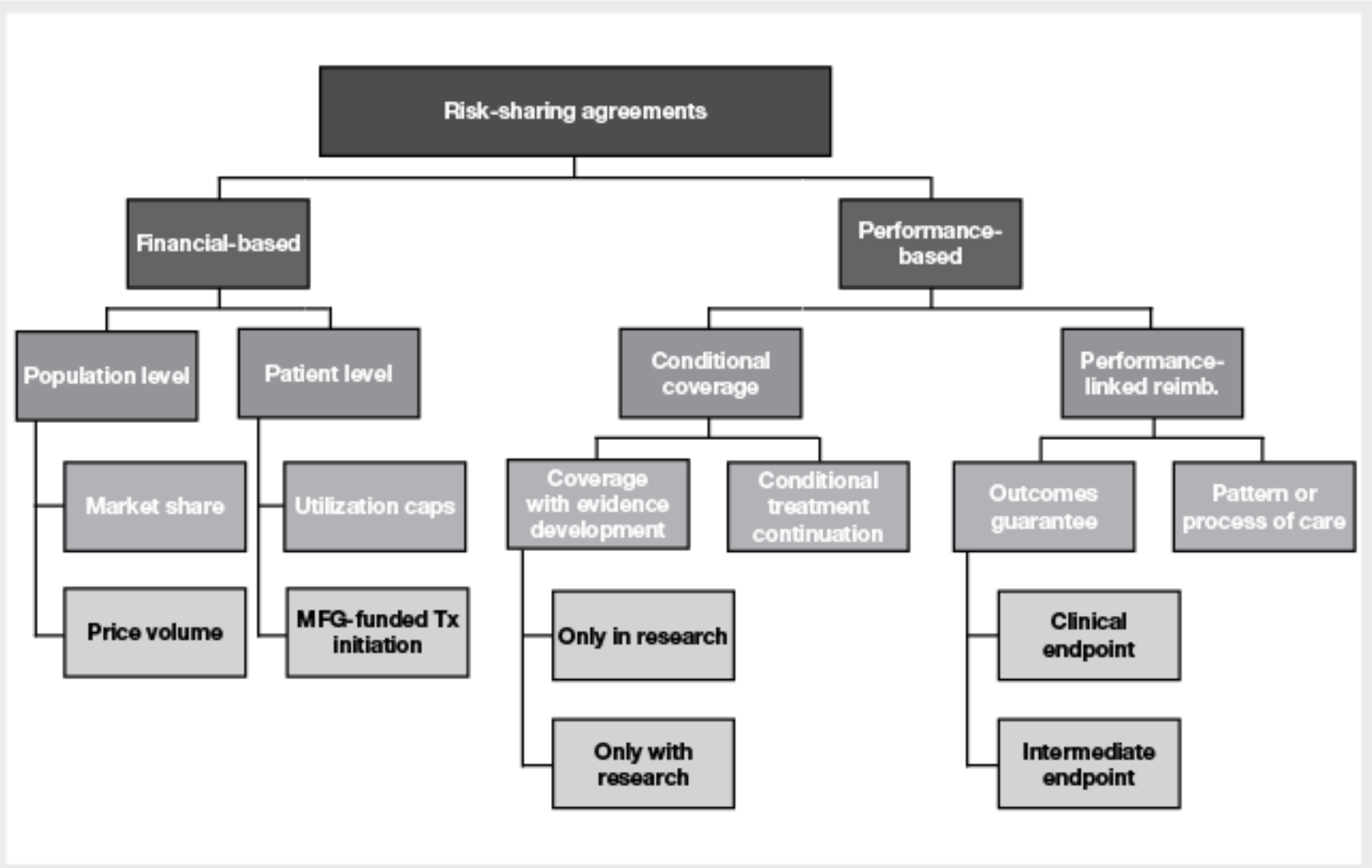


Figure 1. RSA Taxonomy.[7] Abbreviations: MFG, manufacturing; reimb., reimbursement; Tx, treatment.

Partilha de Risco

Table 1: Cap agreement(s) (negotiated and ongoing)

Brand name (active substance)	Manufacturer	Indication	Comments
Perjeta (pertuzumab)	Roche	Metastatic breast cancer HER2 positive	A 2-year contract was signed in June 2014, subject to renewal. The cap is expected to be increased after the first renewal
Zytiga (abiraterone acetate)	Janssen	Metastatic castration- resistant prostate cancer	Already negotiated. No details disclosed for this contract
Kalydeco (ivacaftor)	Vertex	Cystic fibrosis	Already negotiated. No details disclosed for this contract
Incivo (telaprevir)	Janssen	Hepatitis C	Already negotiated. No details disclosed for this contract
Olysio (simeprevir)	Janssen	Hepatitis C	Cap is €25k/patient
Sovaldi (sofosbuvir)	Gilead	Hepatitis C	Cap is €125 million/year and €25k/patient

Journal of Clinical Pathways. 2015;1(1):43–51

Partilha de Risco

Table 1. Financial-Based Patient Access Schemes[13]

Description of Scheme	Drug Product
Providing the first treatment cycle for free	Sutent (sunitinib) Cimiza (certolizumab pegol)
Implementing a payment cap	Revlimid (lenalidomide) Stelara (ustekinumab)
Initial free treatment followed by payment cap to treat responders	Iressa (gefitinib)
Rebate of 16% of amount of drug used on a per patient basis	Erbitux (cetuximab)
Undisclosed discount	Tarceva (erlotinib) Tafinlar (dabrafenib)

Partilha de Risco

Discórdias e Soluções

Type of hurdle	Example of payer pushback	Potential contract solution
Limited budget	"I do not think our budget will allow us to treat the indicated population at that price."	**Price-volume agreement** (cap on payer's total expenditure)
Variable treatment duration	"You say the median duration of treatment is 5 months, but you had a significant portion of patients who were treated for over 7 months. We cannot afford to treat more than 10% of patients beyond 7 months."	**Utilization cap** that limits patient expenditure to 7 months of therapy
Variable dosing	"You say the average dose is 50mg, but dose escalation in this space is very common. We want a price that assumes some level of dose escalation."	**Capitated contract** where the cost per patient is the same regardless of the dose
Adverse events	"Your therapy has some pretty serious adverse events. That could lead to a lot of hospital costs for us."	**Performance guarantee** where the manufacturer covers the costs of the patient's care in the event the patient experiences an adverse event
Delay in patient response	"It takes 3 months to identify a response, and 50% of patients do not respond. That is a lot of money that is just wasted."	**Conditional treatment continuation agreement** where initial treatment is provided for free but payers pay for any continued treatment

Table 1: Hurdles that may be addressed through risk-sharing

Partilha de Risco

Table 4 Interagency agreement on recommendations

Drug	Indication	INESSS	CDR/JODR/pCODR	NICE
Erlotinib	mNSCLC (second–third line)	+	+	+ (with arrangement)
Pemetrexed	mNSCLC (second line)	+	+ (with arrangement)	– (not cost effective)
Pemetrexed	mNSCLC (maintenance)	+	+*	+
Everolimus	mRCC (second line)	+	+ (with arrangement)	+ (with arrangement)
Pazopanib	mRCC (first line)	+	+ (restricted population)	+ (with arrangement)
Sunitinib	mRCC (first line)	+	+ (with arrangement)	+ (with arrangement)
Sorafenib	mRCC (second line after cytokines)	– (not cost effective)	+	– (not cost effective)
Temsirolimus	mRCC (first line poor prognosis)	+	+ (with arrangement)	– (not cost effective)
Axitinib	mRCC (second line)	+ (with arrangement)	+ (restricted population)	– (not cost effective)
Eribulin	mBC (third line)	+	+ (with arrangement)	– (not cost effective)
Cetuximab	mCRC EGFR+ *KRAS* nonmutated	+	+ (with arrangement)	– (not cost effective)
Panitumumab	mCRC EGFR+ *KRAS* nonmutated (second–third line)	– (not cost effective)	+	– (not cost effective)
Imatinib	CML (first line)	+	+	+
Dasatinib	CML (second line)	+	+	– (not cost effective)

Notes: *Based on Cancer Care Ontario guidelines; funded guidelines assumed even if no HTA review.

Abbreviations: CDR, Common Drug Review; CML, chronic myeloid leukemia; EGFR, epidermal growth factor receptor; HTA, health technology assessment; INESSS, Institut National d'Excellence en Santé et Services Sociaux; JODR, Joint Oncology Drug Review; *KRAS, Kirsten rat sarcoma viral oncogene homolog*; mBC, metastatic breast cancer; mCRC, metastatic colorectal cancer; mNSCLC, metastatic non-small-cell lung cancer; mRCC, metastatic renal cell cancer; NICE, National Institute for Health and Care Excellence; pCODR, pan-Canadian Oncology Drug Review.

Chabot I, Rocchi A. Oncology drug health technology assessment recommendations: Canadian versus UK experiences. Clinicoecon Outcomes Res. 2014; 6: 357–367

Programas de acesso precoce e partilha de risco

Obrigado