

美国超说明书用药医保支付管理研究及对我国的启示[△]

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摘要 **目的** 为完善我国超说明书用药的医保支付管理政策提供参考。**方法** 系统梳理美国公共医保中超说明书用药医保支付的制度框架与核心机制, 总结经验并提出对我国超说明书用药医保支付的优化建议。**结果与结论** 美国已建立药品监管与医保支付分工明确的制度框架; 美国FDA负责上市审批和推广行为监管, 医保和医疗补助服务中心(CMS)负责制定报销政策, 将“药品能否使用”与“药品能否支付”相区分。支付管理以动态更新的权威药物数据库为主要依据, 以已发表的医学文献为辅助依据, 构建循证决策基础。CMS通过年度公开申请和评估程序, 对权威药物数据库名单进行动态调整; 同时, 通过事前授权机制, 对高费用、高风险的超说明书用药进行前置审核, 控制医保基金风险。建议我国在临床需求突出的疾病领域开展基于循证的医保支付试点, 依托真实世界研究证据, 选择已有一定循证证据支持的药品探索超说明书用药支付路径; 同时引入事前授权机制, 结合医保信息平台实现标准化审核, 在保障合理用药的同时增强医保基金支出可控性, 维护患者健康权益。

关键词 超说明书用药; 医保基金; 事前授权; 医保支付; 循证决策

Health insurance reimbursement management of off-label drug use in the United States and its implications for China

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ABSTRACT **OBJECTIVE** To provide reference for improving relevant policies of health insurance reimbursement management for off-label drug use in China. **METHODS** The institutional framework and core mechanisms of public health insurance reimbursement for off-label drug use in the United States were systematically reviewed. Experiences were summarized and optimization suggestions for health insurance reimbursement of off-label drug use in China were proposed. **RESULTS & CONCLUSIONS** The United States has established a clearly defined institutional framework with division of responsibilities between drug regulation and health insurance reimbursement: FDA is responsible for marketing approval and promotional regulation, while the Centers for Medicare & Medicaid Services (CMS) is responsible for formulating reimbursement policies, distinguishing between “whether a drug can be used” and “whether a drug can be reimbursed”. Reimbursement management is built on an evidence-based decision-making foundation, primarily relying on dynamically updated authoritative drug compendia, supplemented by published medical literature. CMS dynamically updates the list of authoritative drug compendia through an annual public application and evaluation process. Additionally, prior authorization mechanisms are employed to conduct prospective reviews of high-cost, high-risk off-label drug uses, thereby controlling the risk to health insurance funds. It is recommended that China pilot evidence-based reimbursement for off-label drug use in disease areas with prominent clinical needs, relying on real-world research evidence and selecting drugs with certain evidence-based support to explore reimbursement pathways for off-label drug use. Meanwhile, prior authorization mechanisms should be introduced, combined with health insurance information platforms to achieve standardized reviews, so as to enhance the controllability of health insurance fund expenditures while ensuring rational drug use and safeguarding patients’ health rights and interests.

KEYWORDS off-label drug use; health insurance fund; prior authorization; health insurance reimbursement; evidence-based decision-making

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